ONCOLOGY CENTER OF EXCELLENCE

2025 | OCE Annual Report

VISION

We seek to create a unified and collaborative environment to advance the development and regulation of oncology products for patients with cancer.

MISSION

The mission of the Oncology Center of Excellence (OCE) is to achieve patient-centered regulatory decision-making through innovation and collaboration.

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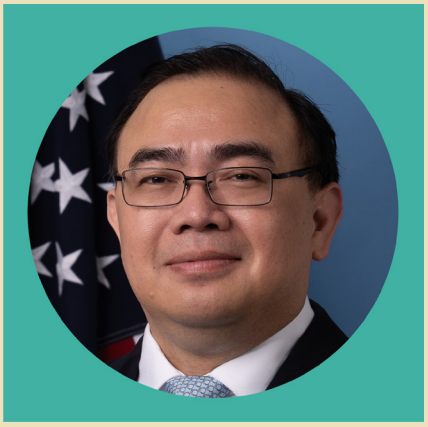
OCE LEADERSHIP

Former Director Dr. Richard Pazdur with
Current Director, Dr. Angelo de Claro

White Oak Campus, FDA

DIRECTOR'S MESSAGE



I am honored to be selected as Director of FDA's Oncology Center of Excellence (OCE) and lead OCE's unwavering commitment to patient-centered regulatory decision making. Having been at FDA for the past 15 years, I've seen major changes at FDA, including new regulations, modernization initiatives, and various reorganizations including changes in leadership. Through it all, FDA staff have supported each other and rose to meet the various challenges over the years. I look forward to working with all of you as we continue the important work and mission of our organization.

As I reflect on the 2025 accomplishments and collaborations in this annual report, it is clear to me that OCE continues to achieve our public health mission and make a meaningful impact to patients. This is due to the institutional resilience of OCE and our review divisions. The collective leadership and expertise ensure that our regulatory excellence continues uninterrupted through periods of change.

OCE continues to innovate and streamline application review without compromising safety and efficacy standards through our current Assessment Aid (AAid) process and Real-Time Oncology Review (RTOR). The OCE Oncology AI program is refocusing its efforts to be regulatory review-oriented and is exploring how AI may impact regulation of oncology drug development. OCE also participated in the Commissioner's National Priority Voucher (CNPV) to review zongertinib for HER2-mutated lung cancer and teclistamab for multiple myeloma. I am committed to building off OCE's experience with AAid and RTOR to pioneer review methodologies that set global standards for regulatory excellence.

International collaboration has been a cornerstone of my work at OCE, and it will remain a key focus as Director. Since 2019, I have had the honor of leading OCE's global clinical sciences program, including coordination of Project Orbis—our groundbreaking initiative that facilitates simultaneous submission and review of oncology marketing applications across multiple regulatory agencies worldwide. I have witnessed firsthand the power of international cooperation in advancing cancer care globally. These partnerships not only expedite access to innovative treatments but also enhance the quality of our regulatory decisions through shared expertise.

“OCE is positioned to continue leading the transformation of cancer care to patients through innovative regulation.”

Looking toward 2026 and beyond, OCE is prepared and stands ready to meet future challenges with confidence. OCE’s foundation is strong with experienced leadership, innovative programs, global partnerships, and an unwavering commitment to our mission. I look forward to engaging the cancer community through external workshops, advisory committees, and expanded international partnerships. Together—as academics, industry, advocates, and global regulatory partners—OCE is positioned to continue leading the transformation of cancer care to patients through innovative regulation.

Sincerely,

R. Angelo de Claro, M.D.
Director
Oncology Center of Excellence

OCE staff on the Quad
White Oak Campus, FDA
2026

REGULATORY REVIEW METRICS

APPROVAL HIGHLIGHTS

Division of Oncology 1

(breast, gynecologic, genitourinary, supportive care)

- Avutometinib and defactinib for KRAS-mutated recurrent, low-grade serous ovarian cancer in adult patients who received prior systemic therapy.
- Datopotamab deruxtecan-dlnk for adult patients with unresectable or metastatic, hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative (immunohistochemistry (IHC) 0, IHC 1+ or IHC 2+/in situ hybridization negative (ISH-)) breast cancer who have received prior endocrine-based therapy and chemotherapy for unresectable or metastatic disease.
- Darolutamide indicated for adult patients with metastatic castration-sensitive prostate cancer.
- Durvalumab with gemcitabine and cisplatin for the peri-operative treatment of patients with muscle-invasive bladder cancer who are undergoing cystectomy. This is the first FDA approval that includes neoadjuvant treatment for this patient population.
- Enfortumab vedotin-ejfv with pembrolizumab or pembrolizumab berahyaluronidase alfa-pmph for the peri-operative treatment of patients with muscle-invasive bladder cancer who are undergoing cystectomy and are ineligible for cisplatin-based chemotherapy. This is the first FDA approval that includes neoadjuvant treatment for this patient population.
- Fam-trastuzumab deruxtecan-nxki for adult patients with unresectable or metastatic hormone receptor (HR)-positive, HER2-low (IHC 1+ or IHC 2+/ISH-) or HER2-ultralow (IHC 0 with membrane staining) breast cancer, as determined by an FDA-approved test, that has progressed on one or more endocrine therapies in the metastatic setting. This is the first approval for HR-positive HER2-ultralow breast cancer.
- Fam-trastuzumab deruxtecan-nxki in combination with pertuzumab is indicated for the first-line treatment of adult patients with unresectable or metastatic HER2-positive breast cancer.

2025 Oncology Approvals*

NEW MOLECULAR ENTITY (NME) OR ORIGINAL BLA APPROVALS	17
RTOR	3
AAID	15
PRIORITY REVIEW	13
REGULAR APPROVAL	9
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AAID	5
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SUPPLEMENT APPROVALS (NEW INDICATIONS)	38
RTOR	3
AAID	28
PRIORITY REVIEW	26
REGULAR APPROVAL	37
ACCELERATED APPROVAL	1

*Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER) oncology approvals.

Number of Oncology Sponsor Meetings Granted in 2025

CDER, CBER	1277

- Gemcitabine releasing system was approved as a drug-device combination that slowly releases a chemotherapy (gemcitabine) into the bladder for a type of non-muscle invasive bladder cancer not responsive to standard of care treatment. This is the first drug-device combination approved to treat this disease.

- Imlunestrant for estrogen-receptor-positive, HER2-negative, estrogen receptor-1-mutated breast cancer in patients whose disease has progressed after at least one endocrine therapy.

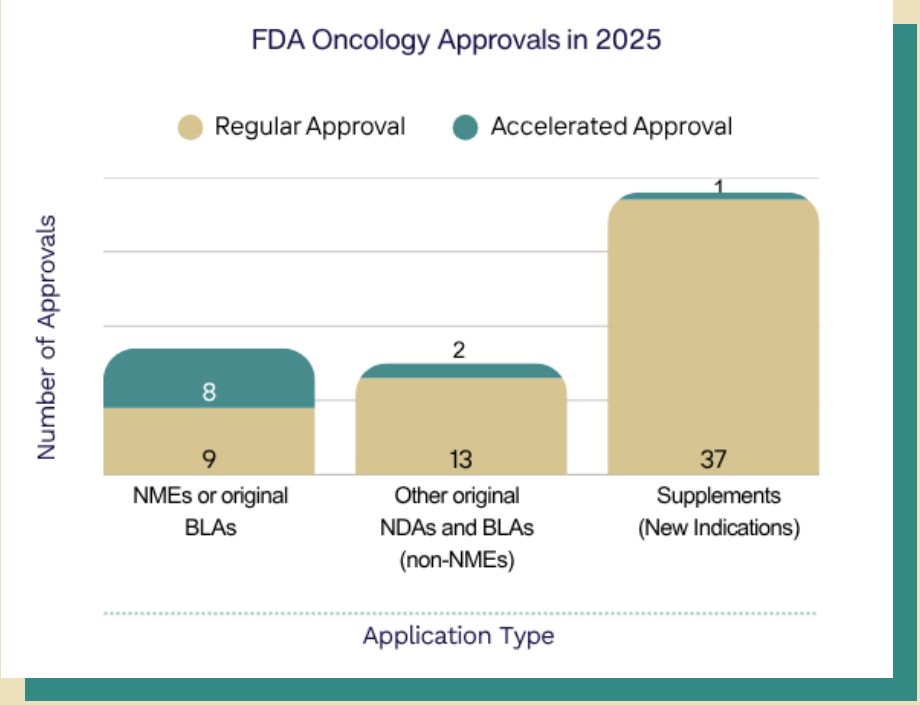
- Lutetium Lu 177 vipivotide tetraxetan for patients with prostate-specific membrane antigen (PSMA)-positive metastatic castration-resistant prostate cancer who have been treated with an androgen receptor pathway inhibitor therapy and are considered appropriate to delay taxane-based chemotherapy. This is the first FDA approval for a radiopharmaceutical prior to chemotherapy.

- Mitomycin was approved in a new formulation for recurrent low-grade, intermediate-risk, non-muscle invasive bladder cancer. The new formulation forms a semi-solid gel in the bladder for sustained drug release, providing a pharmacologic alternative to repeated surgical interventions. This is the first FDA approval indicated specifically for patients with intermediate-risk disease.

- Niraparib and abiraterone acetate for patients with BRCA2-mutated metastatic castration-sensitive prostate cancer. This is the first approval for a poly ADP ribose polymerase (PARP) inhibitor in this early metastatic disease setting.

- Pertuzumab-dpzb is the first biosimilar approval for pertuzumab and did not need a Clinical Endpoint Evaluation (CEE) to demonstrate clinical equivalence in patient outcomes. It is approved for all the same indications as pertuzumab.

- Rucaparib for patients with BRCA-mutated metastatic castration-resistant prostate cancer who have been treated with an androgen receptor-directed therapy.



Division of Oncology 2

(thoracic, head & neck, neuro-oncology, rare, pediatric solid tumors)

- Amivantamab and hyaluronidase-lpuj for subcutaneous injection for adult patients with non-small cell lung cancer (NSCLC) across all indications approved for the intravenous formulation of amivantamab. This FDA approval is clinically important due to the decreased risk of infusion-related reactions (IRR), which is an adverse event after intravenous administration of amivantamab.
- Belzutifan for patients 12 years and older with locally advanced, unresectable, or metastatic pheochromocytoma or paraganglioma. This is the first FDA approval of an oral therapy for paraganglioma.
- Dordaviprone for patients one year and older with diffuse midline glioma (DMG) with the H3K27M mutation and progressive disease following therapy. This is the first FDA-approved systemic therapy for H3K27M mutant diffuse midline glioma.
- Lurbinectedin, in combination with atezolizumab or atezolizumab and hyaluronidase-tqjs for the maintenance treatment of adult patients with extensive-stage small cell lung cancer (ES-SCLC) whose disease has not progressed after first-line induction therapy with atezolizumab and hyaluronidase-tqjs, carboplatin, and etoposide. ES-SCLC is associated with poor survival and is an area of high unmet medical need; this FDA approval was based on an improvement in overall survival.
- Midametinib for adult and pediatric patients two years of age and older with neurofibromatosis type 1 (NF1) who have symptomatic plexiform neurofibromas (PN) not amenable to complete resection.

Dr. Tamy Kim speaking at year-end gathering.
White Oak Campus, FDA
2025

- Pembrolizumab for patients with resectable, locally advanced head and neck squamous cell carcinoma (HNSCC) whose tumors express programmed death-ligand 1 (PD-L1) (combined positive score (CPS) ≥1), as a single agent as neoadjuvant treatment, continued as adjuvant treatment in combination with radiotherapy with or without cisplatin after surgery, and then as a single agent. This is the first FDA approval of a programmed death-1 (PD-1) antibody (or other immunotherapy) as peri-operative therapy for the treatment of patients with resectable HNSCC.

- Sunvozertinib for locally advanced or metastatic NSCLC in patients with epidermal growth factor receptor (EGFR) exon 20 insertion mutations whose disease has progressed on or after platinum-based chemotherapy. This is the only current FDA approval of an orally administered kinase inhibitor for NSCLC with EGFR exon 20 insertion mutations.

- Tarlatamab-dlle for adults with ES-SCLC with disease progression on or after platinum-based chemotherapy. This is the first therapy granted traditional approval by FDA for previously treated ES-SCLC since 1996 and the first therapy granted approval for previously treated ES-SCLC based on demonstration of an improvement in overall survival.

- Telisotuzumab vedotin-tllv, a c-Met-directed antibody and microtubule inhibitor conjugate, for locally advanced or metastatic, non-squamous NSCLC with high c-Met protein overexpression who have received prior systemic therapy. This is a first-in-class approval and is the first therapy approved by FDA for the treatment of NSCLC with c-Met protein overexpression.

- Zongertinib for unresectable or metastatic non-squamous NSCLC with HER2 tyrosine kinase domain (TKD) activating mutations after systemic therapy. This is the first FDA approval of a kinase inhibitor and an orally administered therapy for NSCLC with HER2 TKD activating mutations.

Division of Oncology 3

(gastrointestinal, cutaneous malignancies including melanoma, sarcoma)

- Durvalumab for perioperative treatment of resectable gastric or gastroesophageal junction (GEJ) adenocarcinoma in combination with fluorouracil, leucovorin, oxaliplatin, and docetaxel (FLOT) chemotherapy. Durvalumab reduced recurrences and improved survival in this high risk group of patients with gastric or GEJ cancers.

- Nivolumab in combination with ipilimumab for microsatellite instability-high/mismatch repair deficient (MSI-H/dMMR) advanced colorectal cancer, an uncommon subgroup of patients with this life threatening malignancy.

- Retifanlimab-dlwr with carboplatin and paclitaxel for adults with inoperable locally recurrent or metastatic squamous cell carcinoma of the anal canal (SCAC) or as a single agent, for adults with locally recurrent or metastatic SCAC with disease progression on or intolerance to platinum-based chemotherapy. This is the first modern approval for anal cancer, a rare tumor.

- Vimseltinib for symptomatic tenosynovial giant cell tumors. This is the second product approved by FDA for treatment of this rare tumor.

Division of Hematologic Malignancies 1

(acute leukemias, myelodysplastic syndromes, chronic myeloid leukemia, hematopoietic stem cell transplantation, supportive care for immune effector cell therapies)

- Revumenib for relapsed or refractory acute myeloid leukemia (AML) with a nucleophosmin 1 (NPM1) mutation in adults and pediatric patients one year and older with no satisfactory alternative treatment options. This was the first product to be approved for this subtype of AML.
- Treosulfan in combination with fludarabine as a preparative regimen for allogeneic hematopoietic stem cell transplantation for adult and pediatric patients 1 year of age and older with AML or myelodysplastic syndrome (MDS). This is the first product approved as part of a preparative regimen specifically for patients with AML or MDS.
- Ziftomenib for relapsed or refractory AML with an NPM1 mutation in adult patients with no satisfactory alternative treatment options. This is the second product approved for this subtype of AML in adult patients.

Division of Hematologic Malignancies 2

(lymphoma, chronic lymphocytic leukemia, multiple myeloma, Waldenstrom's macroglobulinemia, AL amyloidosis, other plasma cell disorders)

- Belantamab mafodotin-blmf, a B-cell maturation antigen (BCMA)-directed antibody and microtubule inhibitor conjugate, with bortezomib and dexamethasone for adults with relapsed or refractory multiple myeloma who have received at least two prior lines of therapy, including a proteasome inhibitor and an immunomodulatory agent. The application was discussed at an Oncologic Drug Advisory Committee (ODAC) meeting in July 2025. The application was approved for a revised indication with a Risk Evaluation and Mitigation Strategy (REMS) with Elements to Assure Safe Use (ETASU).
- Brentuximab vedotin in combination with lenalidomide and a rituximab product for adult patients with relapsed or refractory large B-cell lymphoma (LBCL), including diffuse large B-cell lymphoma (DLBCL) not otherwise specified (NOS), DLBCL arising from indolent lymphoma, or high-grade B-cell lymphoma (HGBL), after two or more lines of systemic therapy who are ineligible for autologous hematopoietic stem cell transplantation (auto-HSCT) or chimeric antigen receptor (CAR) T-cell. The first anti-CD30 antibody drug conjugate approved for patients with DLBCL.
- Daratumumab and hyaluronidase-fihj as single agent for adults with high-risk smoldering multiple myeloma (SMM) disorder with a high risk of progression to active multiple myeloma.
- Epcoritamab-bysp with lenalidomide and rituximab for relapsed or refractory follicular lymphoma (FL). This is the first bi-specific antibody combination therapy approved for earlier line FL patients.
- Pirtobrutinib, a non-covalent Bruton's tyrosine kinase (BTK) inhibitor, approved for adults with relapsed or refractory chronic lymphocytic leukemia or small lymphocytic lymphoma (CLL/SLL) who have previously been treated with a covalent BTK inhibitor. This approval was based on improvement in progression free survival in a randomized controlled trial.

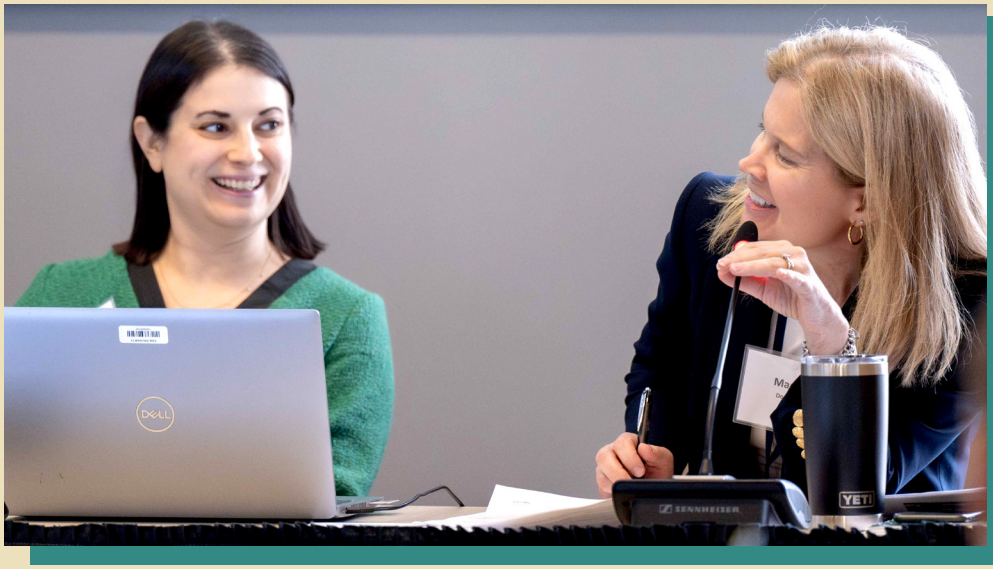
FDA Staff at the entrance of Building 22
White Oak Campus
2025

Center for Devices and Radiological Health

In 2025, the Center for Devices and Radiological Health (CDRH), in collaboration with OCE, authorized more than 90 oncology devices. These authorizations include 26 in vitro diagnostic devices (IVDs), 15 of which were new IVDs with a total of six new companion diagnostic indications, and nine expanded companion diagnostic indications for seven previously authorized companion diagnostic IVDs. Sixty-three (63) radiation oncology and diagnostic radiology devices were authorized as well as seven other oncology-related devices.

CDRH Premarket Submission Highlights Include:

- Approval of two companion diagnostic tests used to identify colorectal cancer (CRC) patients who could benefit from treatment with OPDIVO (nivolumab) as a monotherapy and/or treatment in combination with YERVOY (ipilimumab).
- De novo authorization granted for the MuReva OM, an intraoral phototherapy device granted Breakthrough Device designation and used to treat oral mucositis, a complication in patients receiving radiation treatment with or without chemotherapy.
- De novo authorization granted for Allix5, an Artificial Intelligence/Machine Learning (AI/ML)-based radiological software device intended to generate a five-year risk prediction of breast cancer based on bilateral screening mammogram images.
- Clearance to use the da Vinci SP Surgical System, Model SP1098, and da Vinci SP Instruments to perform robotic nipple sparing mastectomy (NSM) surgical procedures.
- Clearance of multiple orthopedic devices for use in the lower extremities to reconstruct the femur and tibia following resection of primary or metastatic tumors.



OCE Staff at Project Orbis 6 Year Celebration
White Oak Campus, FDA

Pediatric Oncology Program

Project Team: Martha Donoghue, Nicole Drezner, Jennifer Lee, Fatima Rizvi, Pamela Balcazar

The OCE Pediatric Oncology Program (OCE POP) continues to advance development of new safe and effective drugs and biologics to treat cancers in infants, children and adolescents by leveraging the authority to require pediatric studies under the Pediatric Research Equity Act (PREA) and incentivize pediatric development by issuing Written Requests for pediatric studies under the Best Pharmaceuticals for Children Act (BPCA).

The OCE POP also provides community outreach and works with a variety of U.S. and international stakeholders to facilitate timely global pediatric cancer drug development programs.

In 2025, the OCE POP:

- Held six Type F meetings to discuss pediatric study plans for new targeted therapies
- Issued 117 agreed initial pediatric study plans (iPSPs), including 24 with planned pediatric studies
- Issued five Pediatric Written Requests
- Granted pediatric exclusivity for six products with pediatric studies conducted under a Written Request

There were 11 approvals of 12 different drugs and biologics granting 21 new indications to treat pediatric patients with cancer. One particularly notable approval is the approval of dordaviprone, the first FDA-approved systemic therapy for adult and pediatric patients one year of age and older with DMG harboring an H3 K27M mutation. Diffuse intrinsic pontine glioma, a fatal brain cancer that primarily affects school-aged children, is a subtype of DMG. See the OCE Pediatric Oncology Drug Approvals webpage for more information about pediatric oncology drug approvals.

Rare Cancers Program

Project Team: Martha Donoghue, Fatima Rizvi

The Rare Cancers Program's mission is to leverage OCE's ongoing initiatives to promote development of safe and effective new drugs and biologics to treat patients with rare cancers.

Outreach activities in 2025 included an FDA/Osteosarcoma Institute workshop to advance osteosarcoma drug development and a two-day multistakeholder workshop organized by the FDA to help advance use of registries to support oncology drug development. One-day of this workshop focused on use of registries to support product development for DMG, a rare and typically fatal brain tumor.

Of the 17 OCE approvals of novel oncology products in 2025, 9 (53%) were for orphan-designated indications. One particularly notable approval is the approval of a supplemental indication for Lisocabtagene maraleucel for relapsed or refractory marginal zone lymphoma (MZL) after two or more prior lines of therapy. This is the first and only CAR T cell product approved by the FDA for the treatment of this rare cancer.

In 2025, over half (53%) of novel oncology approvals were for orphan drug indications, highlighting a focus on rare diseases.

PATIENT-FOCUSED INITIATIVES

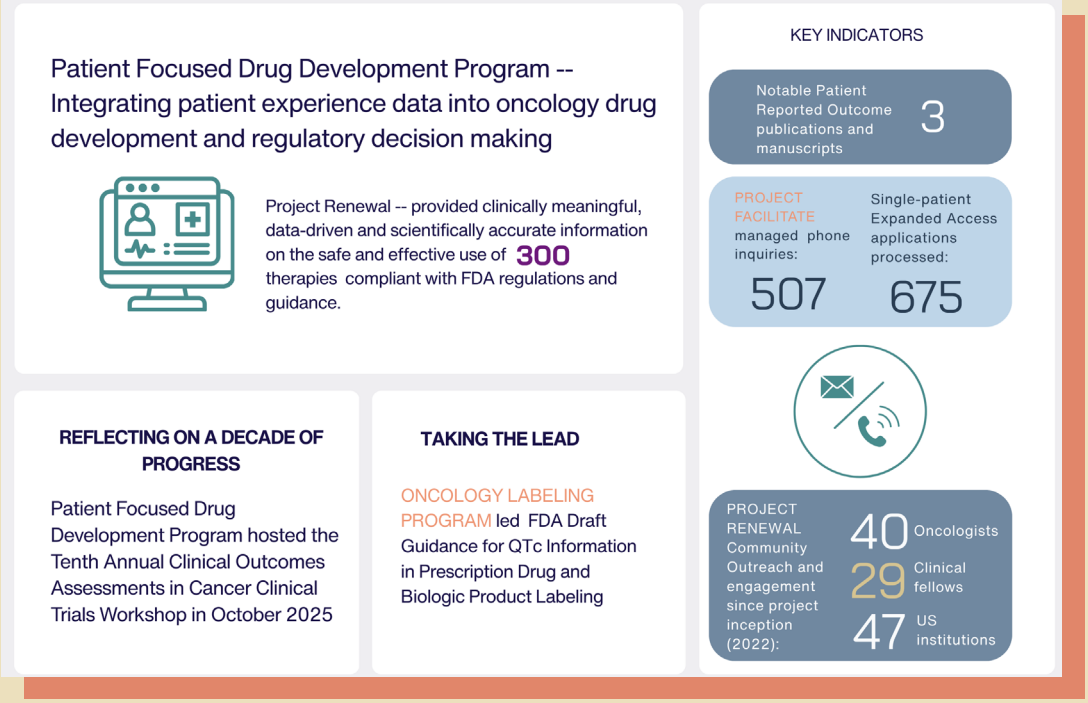
Patient-Focused Drug Development and Project Patient Voice

Project Team: Vishal Bhatnagar, Esther Park, Caitlin Drew

OCE's Patient-Focused Drug Development (PFDD) Program continued its work to integrate patient experience data, including patient-reported symptoms and function, into oncology drug development and regulatory decision making. In 2025, the program produced multiple publications on patient-reported outcomes (PRO) in oncology drug development, including manuscripts based on in-house research as well as funded external research through the OCE Scientific Collaborative. Notable publications include:

- "Assessing tolerability of cancer therapeutics: A regulatory perspective from the FDA Oncology Center of Excellence", which provided the OCE perspective on the evolution of tolerability assessment in oncologic therapeutic evaluation and contextualized research from the National Cancer Institute Tolerability Consortium.
- "Longitudinal graphics of patient-reported physical function in patients treated for hematologic malignancies", which developed analytic methods and visualizations of patient-reported physical function through an estimand framework and iterative stakeholder feedback.
- "Beyond maximum grade: using patient-generated data to inform tolerability of treatments for haematological malignancies", which outlined progress in PRO implementation and future directions including use in early-phase trials and modernization of PRO measures.

The program hosted the Tenth Annual Clinical Outcomes Assessments in Cancer Clinical Trials Workshop in October 2025, titled "Reflecting on a Decade of Progress". The workshop examined the integration of patient-generated data into regulatory submissions over the past decade, discussed research advancing PRO analysis, and analyzed historical examples of core PRO data incorporation into labeling for anti-cancer products. The workshop also explored future directions for PRO data analysis and visualization and identified key achievable milestones for advancing PRO data science and regulatory use over the next five years.



The OCE PFDD program continues to provide clarity and practical advice on measurement of side effects from anti-cancer therapy in all stages of drug development. High-quality patient-reported tolerability data has increasingly impacted FDA's benefit-risk evaluation for drug approvals in oncology, with PRO data more frequently included in oncology product labels, as demonstrated by recent approvals incorporating patient-reported symptomatic adverse events, physical functioning, and overall side-effect bother assessments.

Project Facilitate

Project Team: Tamy Kim, Mitchell Chan, Cam Wilson, Courtney Hamilton

Project Facilitate managed 507 phone inquiries and processed 675 single-patient Expanded Access applications allowing patients with cancer to try investigational medical products when no satisfactory therapies are available and there is no opportunity for the patient to enroll in a clinical trial.

Oncology Labeling Program

Project Team: Doris Auth, Evan Bryson, Adriene King-Ducre, Clara Lee, Barb Scepura

The Oncology Labeling Program collaborates with review teams and efficiently negotiates with applicants to revise oncology product labeling to ensure that they are consistent with FDA regulations and guidances, are clinically meaningful, data-driven and scientifically accurate to facilitate safe and effective use by healthcare providers.

Other notable accomplishments:

- Lead for FDA Draft Guidance for *QTc Information in Prescription Drug and Biologic Product Labeling* (published 12/2/2025)
- Negotiation of revisions of diabetes and hyperglycemia adverse event severity grading from intervention-based grading to laboratory-based grading for National Cancer Institute Common Terminology Criteria for Adverse Events (NCI CTCAE) Version 6.0 for objective and consistent severity information.

Project Renewal

Project Team: Sundeep Agrawal, Doris Auth, Evan Bryson, Clara Lee, Esther Park

Project Renewal brings together FDA scientific staff, practicing oncologists, hematology/oncology fellows in training, oncology pharmacists, and other scientific staff to assess publicly available information to update certain older oncology drugs to ensure information is clinically meaningful and scientifically up to date. Project Renewal continues to refine a repeatable evidence evaluation process in alignment with regulatory standards and labeling requirements while fostering collaboration between FDA and the broader oncology community. OCE will continue to collaborate with CDER Office of Generic Drugs on the MODERN Act to evaluate generic labeling.

In 2025 Project Renewal:

- Organized a public workshop with American Association for Cancer Research (AACR) in January: To Test or Not To Test – That is the Question: DPD Deficiency and Weighing Potential Harms
- Published a Federal Register Notice in May to solicit public comments on dihydropyrimidine dehydrogenase (DPD) deficiency and the use of fluoropyrimidine chemotherapy drugs.
- Facilitated the review of safety labeling changes for Xeloda (capecitabine) with updated information on DPD deficiency.
- Completed FDA independent review of two oncology products.
- Initiated review of three additional oncology products.
- Community outreach and engagement with four oncologists and three fellows (total 40 oncologists and 29 clinical fellows from 47 institutions across the U.S. since program inception).
- Expanded Project Renewal outside of oncology through collaboration with FDA's Division of Anti-Infectives on the standalone Physician Labeling Rule (PLR)/Pregnancy and Lactation Labeling Rule (PLLR) conversion of one anti-infective drug.

INTERNATIONAL COLLABORATION

Project Asha

Project Team: Geetika Srivastava, Abhilasha Nair, Asha Das, Bindu Kanapuru, Fatima Rizvi

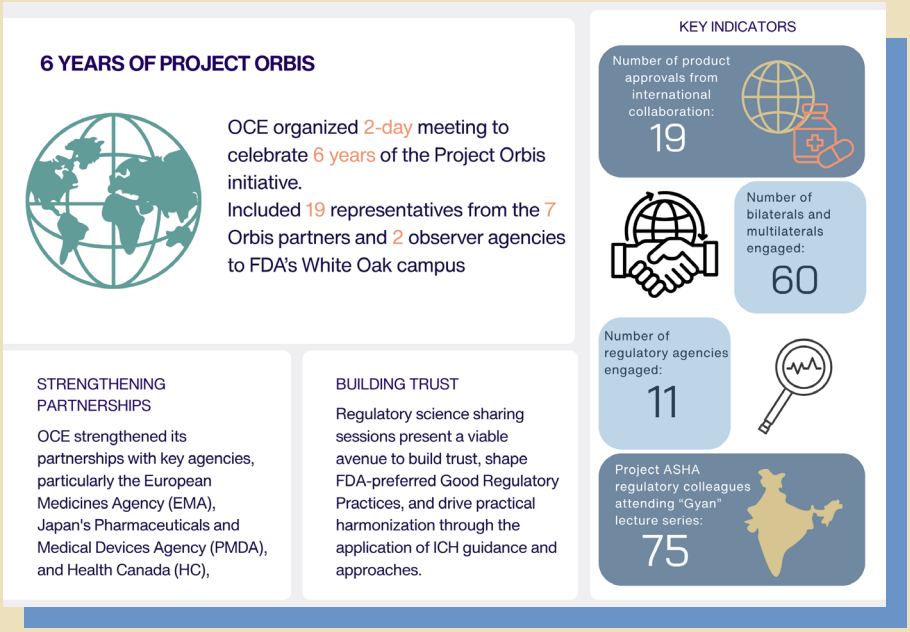
Project Asha is an initiative to increase oncology clinical trial access in India. The goal of this collaboration is to identify short- and long-term opportunities for bilateral cancer cooperation that will drive greater care and improved outcomes for patients with cancer.

In 2025, Project Asha launched a new FDA-CDSCO regulatory lecture series "Gyan", led by FDA reviewers and aimed at the Government of India Central Drug Standard Control Organization (CDSCO) regulators. The first session topic was Chemistry, Manufacturing, and Controls (CMC) review for New Molecular Entities (NME)--small molecules, in which FDA reviewers covered FDA's regulatory science considerations across phases of oncology NME development and review. This marks the first instance of granular, regulatory science practice sharing between FDA and CDSCO's regulatory review divisions. In attendance were over 75 regulatory colleagues from India's CDSCO, encompassing multiple CDSCO review disciplines and functions and led by CDSCO Leadership and the Joint Drugs Controllers of India (CDSCO HQ).

Regulatory science sharing sessions, even without confidential information discussions, present a viable avenue to build trust, shape FDA-preferred Good Regulatory Practices, and drive practical harmonization through the application of International Council for Harmonisation (ICH) guidances and approaches. This enables Project Asha's goals to increase oncology clinical trial access in India through short- and long-term opportunities for bilateral cancer cooperation that will drive greater care and improved outcomes for patients with cancer everywhere.

Project Orbis Partners on FDA campus tour guided by OCE staff.

White Oak Campus, FDA



Project Orbis

Project Team: Dianne Spillman, Angelo DeClaro, Lauren Hotaki, Tina MacAulay, Yinghua Wang, Michael Gu

Project Orbis collaborated with international regulatory partners on 19 product approvals that included three new products and 16 indication extensions. OCE strengthened its partnerships with key agencies, particularly the European Medicines Agency (EMA), Japan's Pharmaceuticals and Medical Devices Agency (PMDA), and Health Canada (HC), advancing programs to support oncology drug development.

Our international engagement continued in 2025 with nearly 60 bilaterals or multilaterals involving 11 different regulatory agencies. These meetings included attending events hosted by other health authorities, such as an EMA-hosted workshop focused on cancer in the elderly, PMDA's Washington DC Office Inauguration, and an EMA forum on dose optimization. In 2025, OCE also launched a quarterly trilateral with EMA and HC to discuss regulatory issues surrounding Cancer in Older Adults. The highlight of the year occurred in June, when OCE organized a two-day meeting to celebrate six years of the Project Orbis initiative. We welcomed 19 representatives from the seven Orbis partners and two observer agencies to FDA's White Oak campus. It was the first time that all the Project Orbis Partners (POPs) were convened in-person, which enabled partnerships to develop among the partners beyond FDA. This meeting was followed by a session at the annual Drug Information Association (DIA) Global where all eight POPs, including FDA, were able to share their experience and perspectives and address questions from stakeholders.

OCE remains committed to fostering these regulatory partnerships, recognizing their crucial role in improving outcomes for patients with cancer worldwide.

STREAMLINING REVIEWS

RTOR

Project Team: Tamy Kim, Jennifer Lee

The RTOR program permits FDA to access topline results and datasets, after datasets are locked, to support an earlier start to the application review. RTOR has enabled the approval of some applications only a few weeks following formal submission. In 2025, the OCE approved four new NDA and BLA applications and three supplemental applications for new indications using the RTOR program.

AAid

Project Team: Tamy Kim, Pamela Balcazar

The AAid is a multidisciplinary review template divided into three parts: The Data, The Applicant's Position, and The FDA's Assessment. This voluntary submission allows FDA's review team to focus their time on critical thinking on the adequacy of the data submitted as well as reducing the amount of time spent on recapitulating information and administrative tasks, such as formatting. In 2025, the OCE approved 20 new NDA and BLA applications and 28 supplemental applications for new indications using the AAid.

Project Point/Counterpoint

Project Team: Dianne Spillman

Project Point/Counterpoint is an initiative that combines the company's and FDA's positions in one ODAC briefing document, similar to the Assessment Aid. This document increases the transparency of differences in viewpoints and concisely focuses on salient data and facilitates the committee's understanding of the critical issues for discussion. Since late 2019, the OCE has recommended the use of this document and has received public support for its use by several standing ODAC members.

In 2025, the ODAC convened for two meetings to discuss a total of five topics. The optional Point/Counterpoint briefing document was used for two of the five topics (40%).

DRUG DEVELOPMENT

Oncology AI Program

Project Team: Jeevan Puthiamadathil, Gautam Mehta, Tamy Kim, Esther Park

The OCE Oncology Artificial Intelligence (AI) Program aims to advance the understanding and application of AI in oncology drug development. The program provides specialized training for reviewers on leading AI methodologies, supports regulatory science research to strengthen AI-related regulatory knowledge, and streamlines the review process for applications that incorporate AI technologies.

In 2025, the program established the AI Council to further advance the understanding and application of AI in oncology drug development. The Council comprises representatives from centers across the Agency, selected for their subject-matter expertise and commitment to scientific innovation. Its primary functions include: providing training on emerging AI approaches, supporting regulatory science initiatives, coordinating the review of oncology-related AI submissions, and implementing process improvements to optimize oncology review operations.

Additionally, OCE has launched pilot programs to develop and validate the use of large language models for regulatory review. In tandem, OCE maintains an internal prompt library to enhance the efficiency of regulatory review.

Project SignifiCanT

Project Team: Rajeshwari Sridhara, Gautam Mehta, Joan Todd, Syed Shah

The Project SignifiCanT (Statistics in Cancer Trials) team, in coordination with the Biopharmaceutical Section of the American Statistical Association and the LUNGevity Foundation, holds discussions with national and international oncology stakeholders to further innovate design and analysis of cancer clinical trials with the goal to advance cancer therapies.

Four key topics discussed in 2025 with multi-disciplinary experts:

1. "Design and Analyses Considerations in the Evaluation of Contribution of Effect in Randomized Cancer Clinical Trials." This forum discussed alternative design and analysis options that could facilitate evaluation of the contribution of effect of each of the components or phases of investigational cancer therapies.

2. “Statistical Considerations for Changes in Standard of Care (SOC) During Ongoing Randomized Cancer Clinical Trials.” This discussion considered trial conduct, incorporation of unplanned adaptations, analyses and interpretation of results when there is a change in available therapy and SOC during an ongoing trial.

3. “Statistical Considerations for Interpreting Duration of Response (DoR) in Cancer Clinical Trials.” This forum considered the utility and interpretation of DoR as an endpoint in cancer clinical trials, including novel methodology for randomized trials.

4. “Statistical Considerations for Hybrid control arms in Cancer Clinical Trials.” Hybrid control arms leverage external or historical data to augment a randomized control arm and may have utility in cancer indications for which conducting a fully powered randomized trial is infeasible. This forum discussion considered the methodology for constructing hybrid control arms in cancer clinical trials.

Project Optimus

Project Team Leads: Mirat Shah, Atiqur Rahman, Pamela Balcazar

Project Optimus is an initiative to reform the oncology drug dosing paradigm. During 2025, Project Optimus achieved significant progress in advancing dosage optimization through its efforts in regulatory review, external education and engagement, and regulatory science.

The project continued to support multidisciplinary review teams across all oncology divisions in providing advice to drug sponsors starting at the pre-IND meeting and continuing through NDA/BLA submission. Project Optimus emphasizes the importance of conducting dosage optimization prior to drug approval. Two NMEs approved in 2025, sunvozertinib and zongertinib, used principles of Project Optimus to conduct premarket randomized dosage evaluations, leading to approval of the lower dose for both products.

The Project Optimus team engaged the external oncology community in multiple forums, contributing expertise on topics including dosage optimization of antibody-drug conjugates, achieving dosage optimization for products on expedited timelines, and principles of dosage optimization for combination products. The Project Optimus team also published numerous articles outlining thinking on critical aspects of dosage optimization, including a series of papers in collaboration with AACR on quantitative aspects of dosage optimization published in Clinical Cancer Research, a perspective on the necessity of dosage optimization for pediatric products published in Clinical Cancer Research, and a manuscript in collaboration with American Society of Clinical Oncology (ASCO) outlining the importance of the totality of data for dosage optimization published in the Journal of Clinical Oncology.

OCE's Global and Regulatory Outreach team welcoming international regulators

FDA White Oak campus.

Project Confirm

Project Team: Gautam Mehta, Fatima Rizvi

Project Confirm enhances transparency around oncology-related Accelerated Approvals through a comprehensive public database. This database, which tracks all oncology Accelerated Approvals since 1992, categorizes approvals as ongoing, converted to traditional approval, or withdrawn. The platform has become an essential resource supporting OCE publications and ODAC meetings.

In 2025, there were eight new oncology accelerated approval indications granted, one new formulation granted accelerated approval for an oncology indication, and 12 oncology accelerated approvals with verified clinical benefit that were "converted" to traditional approval. During this year, Project Confirm supported the activities of the FDA Accelerated Approval Council, reviewed 180-day oncology accelerated approval post-marketing requirement progress reports, and provided internal education on accelerated approval to oncology and broader FDA audiences.

Project Endpoint

Project Team: Nicole Gormley

Project Endpoint aims to enhance the development of endpoints in oncology drug development and foster engagement with the broader community to advance our understanding of and explore potential uses for early, novel endpoints.

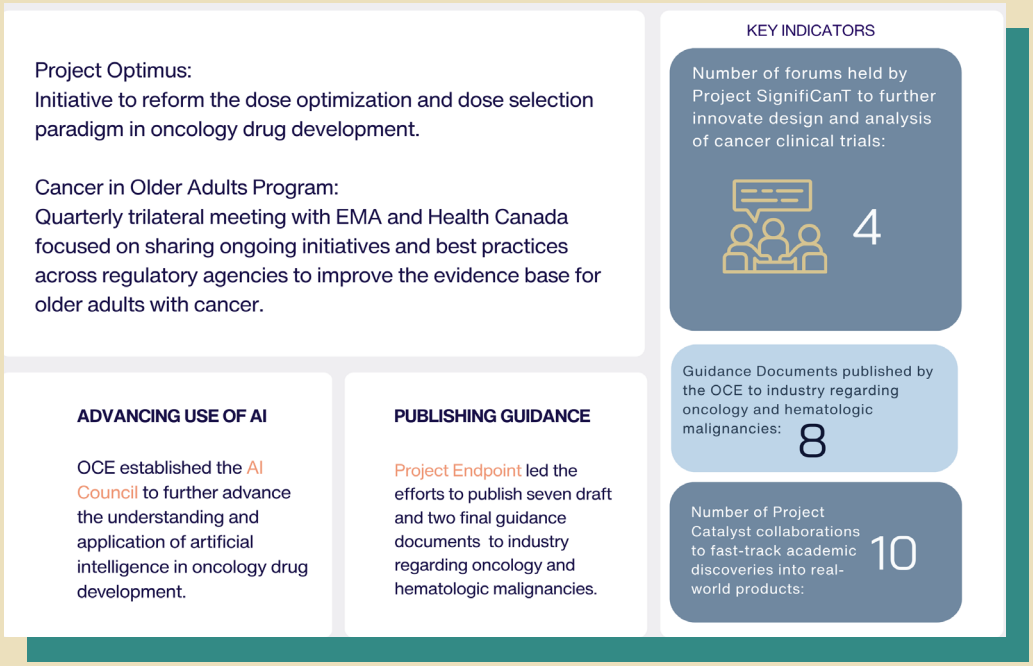
In 2025, Project Endpoint co-hosted a public workshop with AACR in collaboration with Society for Immunotherapy of Cancer (SITC), "Approach to Novel Endpoint Development" to explore considerations for the development and application of novel endpoints in oncology drug development. The workshop discussed previous successful endpoint development approaches, disease areas where novel endpoints are most needed to address existing drug development challenges, and explored strategies to streamline development of novel endpoints among other topics.

Additionally, Project Endpoint led the efforts to publish the draft guidance, *Approaches to Assessment of Overall Survival in Oncology Clinical Trials*. The draft guidance provides recommendations to sponsors on the assessment of overall survival in randomized oncology clinical trials conducted to support marketing approval, with an emphasis on the analysis of overall survival as a pre-specified safety endpoint.

Project Catalyst

Project Team: Jeffery Summers, Joan Todd

Project Catalyst provides guidance and expands educational resources to small pharmaceutical companies and academic life science incubators to support informed anticancer therapy development to the public, including one Academic Accelerator Life Science-hub



Stakeholder Outreach meeting, three Accelerator Innovation Discussion Meetings, one Bench to Bedside Chats, and five Oncology Regulatory Expertise and Early Guidance (OREEG) Program Interactions. All designed to foster collaboration to fast-track academic discoveries into real-world products, drive innovation by providing early regulatory advice and informal scientific discussion.

In coordination with OCE Pediatric and Rare Tumors Program, a Public Private Consortium with FNIH, NIH and FDA was formed to advance the development of therapies for ultra-rare tumors. This new program, the Ultra-Rare Cancer Treatment Advancement (ULTRA) Program will establish a platform and process for development and testing of therapies for ultra-rare cancers for which there is no economic incentives for companies to develop. ULTRA's first design phase meeting was held January 22-23, 2026 – the Target and Treatment Selection Meeting.

Project Catalyst welcomes questions regarding oncology drug development plans that are premature for a pre-IND submission, and values input regarding other efforts that would be useful to early-stage oncology drug development programs.

Oncology RWE Program

Project Team: Vishal Bhatnagar, Diana Bradford, Nicole Drezner, Tamy Kim, Fatima Rizvi

OCE's Oncology Real-World Evidence (RWE) Program, established in 2020, aims to advance the appropriate use of RWE in oncology product development to facilitate patient-centered regulatory decision-making. The program operates across three key areas: regulatory review, regulatory science research, and education and engagement.

OCE Staff at OCE Clinical Rounds.
White Oak Campus, FDA

The RWE Program reviews regulatory submissions containing real-world data (RWD) across oncology review divisions within CDER, CBER, and CDRH. The program maintained active engagement in scientific research with various collaborators and participated in internal meetings and programs across the Centers, including the Advancing RWE Program and CDER's Center for Clinical Trial Innovation.

Cardio-Oncology Program

Project Lead: Laleh Amiri-Kordestani

In 2025, OCE's Cardio-Oncology Program advanced research and regulatory science to improve cardiovascular safety in oncology. The program awarded three competitive research grants supporting innovative approaches, including AI-driven cardiotoxicity risk prediction, echocardiographic biomarker development, and studies of metabolomic and genetic factors underlying immune checkpoint inhibitor–associated cardiovascular toxicity. These projects are expected to enhance early detection and personalized risk stratification, ultimately improving patient outcomes.

The program also contributed to two key publications: an FDA pooled analysis of cardiac adverse events in patients receiving immune checkpoint inhibitors in the adjuvant setting, and a JACC: Cardio-oncology primer on cardiovascular safety in oncology trials. Together, these works provide critical evidence and practical guidance for clinicians, researchers, and regulators.

Additionally, the FDA finalized guidance on "QTc Information in Human Prescription Drug Labeling," strengthening recommendations for incorporating QT interval data into drug labeling. This guidance supports more consistent evaluation and communication of cardiac risk, improving drug safety and informed decision-making.

Cancer in Older Adults Program

Project Team: Bindu Kanapuru, Pamela Balcazar

Adults 65 years and older are a growing segment of the oncology population. Despite advances in treatment of cancers, outcomes for older adults continue to lag their younger counterparts. The Cancer in Older Adults Program is a public health initiative to improve outcomes for older adults with cancer through research, engagement, and regulatory policy. This program serves as a resource for clinicians and scientists, and a focal point for enhancing engagement across FDA's oncology divisions with external stakeholders including patients and global regulatory agencies.

Conversations on Cancer

Empowered Choices: Perspective on Cancer Care Decision-Making Among Older Adults

In 2025, the Cancer in Older Adults Program continued its efforts to engage internal and external stakeholders to address topics pertinent to improving outcomes for older adults with cancer.

In collaboration with Project Orbis, the program initiated a quarterly trilateral meeting with EMA and HC focused on sharing ongoing initiatives and best practices across regulatory agencies to improve the evidence base for older adults with cancer.

Evaluated geriatric section labeling in oncology to develop best practices and improve the safe and effective use of oncology drugs in older patients.

Engaged with patients, advocates, and oncologists through Project Interface-Conversations on Cancer program to discuss the complex milieu of treatment and trial enrollment decisions among older adults with cancer.

Guidance Documents

OCE led the publication of seven draft and two final guidances to industry regarding oncology and hematologic malignancies in 2025. These guidances required collaboration between CDER, CBER, and CDRH.

TITLE	TYPE
QTc Information in Human Prescription Drug and Biological Product Labeling	Final
Technical Specifications for Submitting Clinical Trial Data Sets for Response Assessments for Treatments of Acute Leukemias	Final
Approaches to Assessment of Overall Survival in Oncology Clinical Trials	Draft
Oncology Therapeutic Radiopharmaceuticals: Dosage Optimization During Clinical Development	Draft
Prevention and Treatment of Chemotherapy-Induced Peripheral Neuropathy: Developing Drug and Biological Products in Oncology	Draft
Development of Cancer Drugs for Use in Novel Combination - Determining the Contribution of the Individual Drugs' Effects	Draft
Myelodysplastic Syndromes: Developing Drug and Biological Products for Treatment	Draft
Accelerated Approval and Considerations for Determining Whether a Confirmatory Trial is Underway	Draft
Considerations for Including Tissue Biopsies in Clinical Trials	Draft

ADVANCING EDUCATION AND RESEARCH

Project Socrates

Project Team: Stephanie Wethington, Erin Purcell

Project Socrates welcomed over 140 fellows through workshops and fellowship programs in collaboration with the American Society of Clinical Oncology (ASCO), American Association for Cancer Research (AACR), American Society of Hematology (ASH), American Statistical Association (ASA), and others. This brings the total number of fellows who have participated in these programs to over 1,210.

Project Socrates continued to strengthen and broaden its collaborations by designing new educational initiatives. During 2025, planning began for two inaugural workshops to take place in 2026: the Workshop for Experienced Oncology Patient Advocates and the FDA-American Society of Pediatric Hematology/Oncology Educational Program for early career pediatric oncologists and researchers.

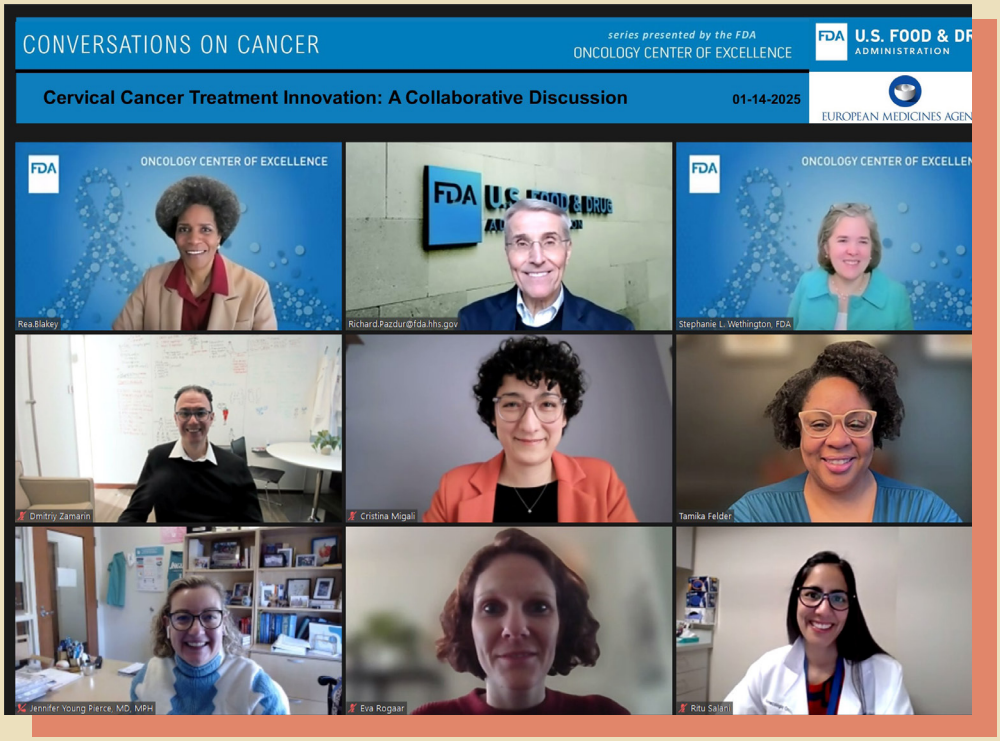
OCE also hosted external speakers such as Drs. Allen Lichter and Keith Flaherty, President Elect of AACR, and hosted internal educational and professional development programing for staff. The OCE Regulatory Science Online Lecture Series added three new episodes, all of which are freely available with accompanying audio and slides.

Project Interface

Project Team: Ginneh Stowe, Nabeel Babaa, Sherwin Sapasap

Project Interface is the outreach and engagement initiative for OCE, supporting and advancing public health priorities related to oncology. Through this initiative, OCE connects with domestic stakeholders, including patients diagnosed with cancer, patient advocacy organizations, faith-based groups, professional societies, and oncology experts to strengthen engagement across the oncology community.

In 2025, Project Interface met with ten organizations to discuss key challenges and opportunities in oncology patient care. These organizations represent stakeholders who cover issues ranging from colorectal care to cancer in Appalachia. These engagements reflect OCE's continued commitment to increasing awareness about cancer and ensuring patient perspectives inform OCE's work.



OCE Conversations on Cancer

Cervical Cancer Treatment Innovation:
A Collaborative Discussion

OCE Scientific Collaborative

Project Team: Rebekah Zinn

The OCE Scientific Collaborative promotes regulatory science research in oncology. FDA oncology staff participate in both intramural and extramural research projects to address specific challenges encountered during the regulatory review processes.

Program highlights from 2025 include:

- Released Request for Applications on Cardiotoxicity of Oncology Therapeutics (FDA-FD-25-015), which resulted in three awards:
- Metabolomic and Genetic Factors Decoupling Immune Checkpoint Inhibitor Tumor Efficacy and Cardiovascular Toxicity (MD Anderson Cancer Center)
- AI-Enabled Echocardiographic Biomarkers and Real-World Data for Predicting Cancer Therapy-Related Cardiac Dysfunction (Kaiser Foundation Research Institute)
- CardioOnco-AI: AI-Empowered Cardiotoxicity Risk Prediction Among Breast Cancer Survivors Using Multi-Site Real-World Data (University of Minnesota)

OCE Staff at ASCO Fellows Day
ASCO Headquarters
September 25, 2025

- Funded two new applied research projects through the Broad Agency Annoucement (BAA) program:
- Statistical Designs and Considerations for Dose Optimization in Drug Combinations and Multiple Indications (MD Anderson Cancer Center)
- Application of Computable Phenotyping to Real-World Data to Support the Active Safety Surveillance of CAR T-cell Therapies (Johns Hopkins University)
- Launched Project Collaborate Crowdsourcing Initiative that built on a pilot effort in 2023 to provide an opportunity for external scientists to submit research questions that could be addressed by pooled analyses of clinical trial data submitted to FDA for regulatory purposes. Eleven submissions were received and are currently under review. In the first campaign, one of the ideas was selected for a collaborative research project, and an abstract was presented at AACR in April 2025.

Advancing Internal Education

Project Team: Marc Neilson

OCE's internal education efforts extended to all centers and divisions through ten core curriculum scientific mastery courses. These courses were aimed at regulators and scientists to improve the impact of their data-specific presentations, external presentations, and information organization within teams. Twenty-four sessions were given to over 100 staff in the Offices of New Drugs, Generic Drugs, Translational Sciences, Executive Leadership, and Regulatory Policy.

OCE 2025 Workshops and Events

NAME	TOPIC	DATE
Conversation on Cancer	Cervical Cancer Treatment Innovation: A Collaborative Discussion	Jan 14
FDA-AACR Workshop	To Test or Not To Test - That is the Question: DPD Deficiency and Weighing Potential Harms	Jan 16
OCE Regulatory Science Research Exchange	Clinical Implementation of Liquid Biopsies in Precision Immuno-Oncology	Jan 17
Icons in Oncology	Dr. Allen Lichter	Feb 3
OCE Regulatory Science Research Exchange	Clinical Trial Endpoints for Higher-Risk Myelodysplastic Syndromes (MDS): Trial-Level and Patient-Level Analyses	Feb 14
Mini-Symposia	Considerations for Optimizing Clinical Trials for Novel Therapies for Metastatic Cutaneous Melanoma	Mar 7
Mini-Symposia	Design and Analyses Considerations in the Evaluation of Contribution of Effect in Randomized Cancer Clinical Trials	Apr 8
OCE Regulatory Science Research Exchange	Cardiac Adverse Events with Immune Checkpoint Inhibitors, A Pooled Analysis	Apr 25
ODAC	(4 topics) glofitamab, daratumumab + hyaluronidase, UGN-102, and talazoparib	May 20-21
Mini-Symposia	B-ALL in 2035 – Where are we going and how do we get there?	May 28
Conversations on Cancer	Beyond the Disease: Religious Literacy and Spirituality in Cancer Care	Jun 10
OCE Workshop	Project Orbis Six-Year Celebration	Jun 12-13

OCE 2025 Workshops and Events (cont.)

NAME	TOPIC	DATE
OCE Regulatory Science Research Exchange	Closing the Clinical Trials Efficacy Measurement Gap: Novel Methodologies for Assessing Response to Cancer Treatment in Real-World Studies	Jun 13
DIA Global	Project Orbis: Six Years Later	Jun 16
DIA Global	Setting the Bar High: Facilitating Quality and Acceptability of Decision-Grade Real-world Evidence	Jun 16
DIA Global	Regulatory Cooperation Between US and Japan	Jun 17
DIA Global	Harmonizing Data Quality Frameworks: Bridging Regulatory Perspectives	Jun 17
Mini-Symposia	Neuroblastoma minor responses in the relapsed and refractory setting	Jun 17
DIA Global	Modernizing Evidence in Oncology: Real-World Data and Artificial Intelligence in Clinical Drug Development	Jun 18
OCE Regulatory Science Research Exchange	Multiregional Clinical Trials and U.S. Patient Enrollment in Pivotal Clinical Trials	Jul 11
ODAC	Belantamab mafodotin	Jul 17
FDA Public Workshop	The Future of Registries in Oncology: Best Practices for Innovation in Drug Development Virtual Public Workshop	Aug 27-28
FDA-AACR Workshop	Approach to Novel Oncology Endpoint Development Workshop	Sep 11
Conversations on Cancer	Endometrial Cancer: Rising Incidence. Rising Innovation	Sep 18

OCE 2025 Workshops and Events (cont.)

NAME	TOPIC	DATE
OCE Regulatory Science Research Exchange	Patient-Focused Drug Development Research Update	Sep 19
OCE Workshop	FDA-ASH Collaboration: A Workshop on Regulatory Science in Hematology	Sep 29-30
10th Clinical Outcome Assessment in Cancer Clinical Trials Workshop	Reflecting on a Decade of Progress	Oct 8
FDA-Osteosarcoma Institute Workshop	Advancing Osteosarcoma Drug Development – Connecting Research and Regulatory Pathways for Improved Outcomes	Oct 10
FDA-ASCO Leadership Development Program Meeting	Leadership in Government - A Non-Oncology Course for Oncologist	Oct 15
OCE Regulatory Science Research Exchange	Immunotherapy Outcomes in Patients of Native American Ethnicity: A Multicenter Retrospective Study	Oct 24
OCE Regulatory Science Research Exchange	Modeling Pediatric Solid Tumors and the Tumor Microenvironment	Nov 21
Mini-Symposium	PFS2 as Clinical Trial Endpoint: Regulatory Considerations	Dec 4
Conversation on Cancer	Empowered Choices: Perspectives on Cancer Care Decision-Making Among Older Adults	Dec 11

OCE Publications

OCE's strong support for publications by FDA oncology/hematology staff continued in 2025, resulting in 46 articles in scientific journals. This included 10 research papers, 19 FDA approval summaries, and 17 regulatory perspectives.

Research Papers

1. Most Common Symptomatic Adverse Reactions of Cancer Treatments From US Drug Labels (2015-2021) to Inform Selection of Patient-Reported Outcomes. Horodniceanu EG, Datla T, Murugappan MN, Kanapuru B, Amiri-Kordestani L, Larkins E, Kluetz P, Bhatnagar V. Value Health. 2025 Jan;28(1):108-115. doi: 10.1016/j.jval.2024.09.009. PMID: 39389354.

2. Adoption of Decentralized Trial Elements in Cancer Clinical Trials Supporting FDA Approvals During COVID-19. Patel TH, Corneli A, Balcazar P, Lipset C, Calvert SB, Mervin-Blake S, Nalawade V, Kluetz PG. Clin Cancer Res. 2025 Mar 4. doi: 10.1158/1078-0432.CCR-24-3357. PMID: 40036170.

3. Challenges in Automating Extraction of Real-World Radiographic Images and Adverse Events: Lessons from the ICARE data Initiative. Piantadosi S, Campbell N, Chow S, Elrahi C, Knopp MV, Kumar V, Lerro CC, Rivera DR, Kluetz PG, Quina A, Casagni M, Mohammed-Rajput N, Tevaarwerk A, George S. JCO Clin Cancer Inform. 2025 Apr;9:e2400319. doi: 10.1200/CCI-24-00319. PMID: 40249879.

4. Cardiac Adverse Events in Patients Receiving Immune Checkpoint Inhibitors in the Adjuvant Setting: An FDA Pooled Analysis. Dilawari A, Krantz MJ, Bulatao I, Joeng HK, Neilson M, Wedam S, Gao X, Fiero MH, Nair A, Theoret M, Amiri-Kordestani L. Ann Noninvasive Electrocardiol. 2025 May;30(3):e70087. doi: 10.1111/anec.70087. PMID: 40343390; PMCID: PMC12059289.

5. FDA pooled analysis of OS according to depth of response in frontline advanced IO RCC trials. Chang E, Gittleman H, Song C, Bloomquist E, Fernandes L, Weinstock C, Agrawal S, Gormley N, Tang S, Suzman DL, Amiri-Kordestani L, Pazdur R, Kluetz PG, McDermott DF, Regan MM, Rini BI. JNCI Cancer Spectr. 2025 Jul 16:pkaf069. doi: 10.1093/jncics/pkaf069.

6. Analysis of Regulatory Botanical Submission Profile for Cancer Management from the U.S. FDA Perspectives. Park JK, Lee D, Rui L, Gao X, Furness MS, Wu C. Ther Innov Regul Sci. 2025 Jun 4. doi: 10.1007/s43441-025-00786-y. PMID: 40468097.

7. Longitudinal graphics of patient-reported physical function in patients treated for hematologic malignancies.Thanarajasingam G, Bhatnagar V, Noble BN, Chen TY, Fiero MH, Hoffman R, Jeffery M, Mazza GL, Mascarenhas J, Mesa R, Murugappan M, Ross J, Sidana S, Warsame R, Kluetz PG, Dueck AC. BMC Med Res Methodol. 2025 Aug 7;25(1):189. doi: 10.1186/s12874-025-02617-y. PMID: 40775758; PMCID: PMC12329971.

8. Reply to: Decoding the End Points of Poly (ADP-ribose) Polymerase Inhibitor Trials in Ovarian Cancer. Shah M, Fiero MH, Cheng J, Chen TY, Ison G, Pazdur R, Amiri-Kordestani L. J Clin Oncol. 2025 Oct 31:JCO2501933. doi: 10.1200/JCO-25-01933. PMID: 41172234.

9. Machine Learning reveals distinct T-cell receptor clusters in plasma cell dyscrasias compared to healthy controls. Coffey DG, Zhang Y, Hill E, Cross F Jr, Philip R, Theoret MR, Landgren O, Baines AC, Kazandjian D. PLoS One. 2025 Oct 27;20(10):e0334053. doi: 10.1371/journal.pone.0334053. PMID: 41144424; PMCID: PMC12558469.

OCE Staff at ASCO Fellows Day
ASCO Headquarters
September 25, 2025

OCE staff at year-end gathering
White Oak Campus, FDA
2025

10. Real-World Outcomes and Treatment Patterns in Patients With Acute Myeloid Leukemia and TP53 Gene Mutation or 17p Deletion. Bystrom R, Fernandes LL, Wynne J, Pulte D, Hansen E, Belli AJ, Barcellos A, Zettler CM, Vallejo J, Gu W, DeClaro A, Lerro CC, Wang CK, Rivera DR, Norsworthy KJ. Am J Hematol. 2025 Dec 13. doi: 10.1002/ajh/70165. PMID: 41388784.

Approval Summaries

1. FDA approvals in 2024: new options for patients across cancer types and therapeutic classes. Agrawal S, Park E, Kluetz PG. Nat Rev Clin Oncol. 2025 Apr 11. doi: 10.1038/s41571-025-01018-w. PMID: 40216937.

2. FDA Approval Summary: Tovorafenib for Relapsed or Refractory BRAF-altered Pediatric Low-Grade Glioma. Singh S, Bradford D, Chatterjee S, Li X, Aungst SL, Skinner AM, Miller CP, Kim-McOlash S, Fourie Zirkelbach J, Xiong Y, Bi Y, Wang YH, Yang Y, Sun J, Kraft J, Charlab R, Shord SS, Tang S, Scepura B, Bulatao I, Udoka O, Saber H, Rahman NA, Pazdur R, Singh H, Donoghue M, Drezner N. Clin Cancer Res. 2025 Jan 14. doi: 10.1158/1078-0432.CCR-24-3439. PMID: 39808502.

3. FDA Approval Summary: Obecabtagene Autoleucel for B-Cell Acute Lymphoblastic Leukemia. Bouchkouj N, Przepiorka D, Fashoyin-Aje LA. JAMA. 2025 Feb 10. doi: 10.1001/jama.2024.28312. PMID: 39928344.

4. FDA Approval Summary: Nadofaragene Firadenovec-vncg for Bacillus Calmette-Guérin-Unresponsive Non-Muscle-Invasive Bladder Cancer. Colbert L, Jia Y, Sharma A, Hu J, Xu Z, Suzman DL, Das A, Bross P, Kluetz PG, Fashoyin-Aje LA. Clin Cancer Res. 2025 Apr 1;31(7):1182-1185. doi: 10.1158/1078-0432.CCR-24-2812. PMID: 39705065; PMCID: PMC11961324.

5. FDA Approval Summary: Afamitresgene Autoleucel for Adults With HLA-Restricted MAGE-A4 Positive Unresectable or Metastatic Synovial Sarcoma After Prior Chemotherapy. Barnett KK, Johnson AR, Das A, Lee CJ, Wang C, Wang X, Cho ES, Kluetz PG, Fashoyin-Aje LA. Clin Cancer Res. 2025 May 27. doi: 10.1158/1078-0432.CCR-25-0595. PMID: 40423661.

6. Remestemcel-L-rknd for Steroid-Refractory Acute Graft-vs-Host Disease in Pediatric Patients. Mahat U, Przepiorka D, Fashoyin-Aje LA. JAMA. 2025 May 21. doi: 10.1001/jama.2025.6179. PMID: 40397429.

7. FDA Approval Summary: Abatacept for prophylaxis of acute graft versus host disease. Norsworthy KJ, Rivera DR, Wynne J, Zhao J, Konicki R, Kuzucan A, Vallejo J, Leong R, Okusanya OO, Booth B, Kluetz PG, Pazdur R, de Claro RA. Clin Cancer Res. 2025 May 28. doi: 10.1158/1078-0432.CCR-25-0688. PMID: 40435096.

8. FDA Approval Summary: Idecabtagene Vicleucel for the Treatment of Triple-Class Exposed, Relapsed or Refractory Multiple Myeloma. Sharma P, Lin X, Xu Z, Kanapuru B, Theoret MR, Sokolic R, Fashoyin-Aje LA. Clin Cancer Res. 2025 Jun 4. doi: 10.1158/1078-0432.CCR-24-4181. PMID: 40465403.

9. FDA Approval Summary: Axatilimab for Adult and Pediatric Patients Weighing at Least 40 Kilograms with Chronic GVHD after Two Prior Lines of Systemic Therapy. Le RQ, Godder K, Wang J, Collazo JS, Konicki R, Choe M, Feng M, Przepiorka D, Vallejo J, Shah A, Liu J, Gehrke BJ, Wilson W, Siegel A, Wu Y, Kuo CY, Ray M, Pazdur R, Theoret MR, de Claro RA. Clin Cancer Res. 2025 Jun 27. doi:10.1158/1078-0432.CCR-25-0896. PMID: 40577088.

10. FDA Approval Summary: Ivosidenib for Treatment of Adult Patients with Relapsed/Refractory Myelodysplastic Syndrome with an IDH1 Mutation. Woods AC, Pulte ED, Wang X, Vallejo J, Chadda R, Zheng N, Blanco JG, Dorff SE, Li H, Liu J, Okusanya OO, Pazdur R, Theoret MR, de Claro RA, Norsworthy KJ. Clin Cancer Res. 2025 Jul 1. doi: 10.1158/1078-0432.CCR-25-1005. PMID: 40590836.

11. US Food and Drug Administration Approval Summary: Trastuzumab Deruxtecan for the Treatment of Adult Patients With Hormone Receptor-Positive, Unresectable or Metastatic Human Epidermal Growth Factor Receptor 2-Low or Human Epidermal Growth Factor Receptor 2-Ultralow Breast Cancer. Dilawari A, Zhang H, Shah M, Gao X, Fiero M, Bhatnagar V, Pierce W, Mixter B, Pazdur R, Amiri-Kordestani L. J Clin Oncol. 2025 Aug 5:JCO2500812. doi: 10.1200/JCO-25-00812. PMID: 40763319.

12. US Food and Drug Administration Approval Summary: Ribociclib With an Aromatase Inhibitor in the Adjuvant Hormone Receptor-Positive, Human Epidermal Growth Factor Receptor 2-Negative Stage II and III High-Risk Early Breast Cancer Treatment Setting. Gao JJ, Prowell TM, Gittleman H, Cheng J, Fiero M, Bulatao I, Ching-Jey Chang G, Ricks TK, Green F, Wu H, Yu J, Grimstein M, Yang Y, Zhao H, Liu Q, Kolhatkar R, Gao T, Tu CM, Hou S, Redwood S, Fuller B, Griffiths L, Ajua-Alemanji M, Leach C, Woods S, Akinboro O, Narayan P, Shah M, Osgood C, Raghavachari R, Rahman A, Tang S, Bhatnagar V, Gormley N, Kim T, Pierce WF, Pazdur R, Kluetz PG, Amiri-Kordestani L. J Clin Oncol. 2025 Aug 11:JCO2500167. doi: 10.1200/JCO-25-00167. PMID: 40789109.

13. FDA Approval Summary: Nogapendekin Alfa Inbakicept-pmln with BCG for BCG-unresponsive carcinoma in situ. Heiss BL, Chang E, Joeng HK, Fiero MH, Wang L, Hamed SS, Chiu HJ, Ricks TK, Koh EH, Dellibovi-Ragheb TA, Wang M, Lee C, Pierce WF, Leighton JK, Rahman NA, Tang S, Pazdur R, Amiri-Kordestani L, Kluetz PG, Suzman DL. Clin Cancer Res. 2025 Aug 18:10.1158/1078-0432.CCR-25-1231. doi: 10.1158/1078-0432.CCR-25-1231. PMID: 40824697; PMCID: PMC12363661.

14. FDA Approval Summary: Blinatumomab for the Treatment of B-cell Precursor Acute Lymphoblastic Leukemia in the Consolidation Phase of Multiphase Chemotherapy. Rabik CA, Wang S, Chadda R, Przepiorka D, Vallejo J, Jiang X, Theoret MR, de Claro RA. Clin Cancer Res. 2025 Aug 19:10.1158/1078-0432.CCR-25-1034. doi: 10.1158/1078-0432.CCR-25-1034. PMID: 40828516; PMCID: PMC12367060.

15. FDA Approval Summary: Lisocabtagene Maraleucel for Relapsed or Refractory Follicular Lymphoma. Britton K, Mahat U, Richardson NC, Tegenge M, Gao Q, Theoret MR, Fashoyin-Aje LA. Clin Cancer Res. 2025 Jul 15:10.1158/1078-0432.CCR-25-1035. doi: 10.1158/1078-0432.CCR-25-1035. PMID: 40663365; PMCID: PMC12369282.

Project Orbis Partners on FDA campus tour guided by OCE staff.

White Oak Campus, FDA

16. US Food and Drug Administration Approval Summary: Inavolisib With Palbociclib and Fulvestrant for Endocrine-Resistant, PIK3CA-Mutated, Hormone Receptor-Positive, Human Epidermal Growth Factor Receptor 2-Negative, Locally Advanced or Metastatic Breast Cancer. Wedam S, Narayan P, Gittleman H, Cheng J, Bhatnagar V, Sabit H, Price LSL, Rahman NA, Chiu HJ, Biel N, Ricks T, Fiero M, Tang S, Osgood C, Pierce W, Pazdur R, Kluetz PG, Amiri-Kordestani L. J Clin Oncol. 2025 Aug 22:JCO2500663. doi: 10.1200/JCO-25-00663. PMID: 40845250.

17. FDA Approval Summary: Datopotamab deruxtecan-dlnk for treatment of patients with unresectable or metastatic, HR-positive, HER2-negative breast cancer. Royce M, Shah M, Zhang L, Cheng J, Bonner MK, Pegues M, Miller CP, Leu L, Price LSL, Qiu J, Yu J, Truong TM, Dorff SE, Yang Y, Zhang N, Gutierrez-Lugo M, Ricks TK, Pierce WF, Luo Z, Kappel D, Goldberg KB, Shord SS, Tang S, Bhatnagar V, Pazdur R, Kluetz PG, Amiri-Kordestani L. Clin Cancer Res. 2025 Aug 27:10.1158/1078-0432.CCR-25-1388. doi: 10.1158/1078-0432.CCR-25-1388. PMID: 40864501; PMCID: PMC12393668.

18. FDA Approval Summary: Vorasidenib for IDH-mutant Grade 2 Astrocytoma or Oligodendroglioma following surgery. Barbato MI, Barone AK, Aungst SL, Miller CP, Ananthula S, Bi Y, Yang Y, Li X, Xiong Y, Fan J, Dorff SE, Zhao H, Zhou H, Pradhan S, Scepura B, Sinha AK, Stephenson M, Bhatnagar V, Saber H, Rahman NA, Tang S, Pazdur R, Kluetz PG, Larkins E, Drezner N. Clin Cancer Res. 2025 Sep 5. doi: 10.1158/1078-0432.CCR-25-1333. PMID: 40911439.

19. US Food and Drug Administration Approval Summary: Imetelstat for Selected Patients With Low- to Intermetiate-1 Risk Myelodysplastic Syndromes With Transfusion-Dependent Anemia. Kim N, Pulte ED, Ehrlich LA, Ionan AC, Haupert S, Vallejo J, Green F, Zheng N, Wang Y, Liu J, Blanco JG, Dorff SE, Booth B, Choe M, Gehrke B, Bhatnagar V, Theoret M, Pazdur R, De Claro RA, Norsworthy KJ, J Clin Oncol. 2025 Oct 24:JCO2501369. doi: 10.1200/JCO-25-01369. PMID: 41135032.

Regulatory Perspectives

1. Real-World Evidence in New Drug and Biologics License Application Approvals During Fiscal Years 2020-2022. Innes GK, Smith KA, Kuzucan A, Li J, Rivera D, Panagiotou OA, Concato J. Clin Pharmacol Ther. 2025 Apr 25. doi: 10.1002/cpt.3688. PMID: 40276902.

2. Increasing the Utility of Real-World Data to Inform Public Health Decision Making Through a US-based Private-Public Partnership: 10 Lessons Learned from a Principled Approach to Rapid Pandemic RWE Generation. Chakravarty A, Rivera D. Ther Innov Regul Sci. 2025 Mar 18. doi: 10.1007/s43441-025-00748-4. PMID: 40102361.

3. Overall Survival and the Evolving Benefit-Risk Assessment for Poly (ADP-ribose) Polymerase Inhibitors in Advanced Ovarian Cancer. Shah M, Chen TY, Ison G, Fiero MH, Zhang H, Gao X, Neilson M, Goldberg KB, Nair A, Ricks TK, Pierce WF, Gormley N, Theoret MR, Tang S, Pazdur R, Kluetz PG, Amiri-Kordestani L. J Clin Oncol. 2025 May 19:JCO2402834. doi: 10.1200/JCO-24-02834. PMID: 40388682.

4. Use of patient-reported outcomes to inform symptom and functional outcomes in cancer drug regulatory decisions: challenges and future directions. Bhatnagar V, Kluetz P, et al. The Lancet Oncology. April 14.

5. Using patient-reported outcomes and health-related quality of life data in regulatory decisions on cancer treatment: highlights from an EMA-EORTC workshop. Bhatnagar V, Kluetz P, et. al. The Lancet Oncology. April 14

6. Defining the role of pragmatic clinical trials in cancer clinical research: outcomes of a collaborative workshop hosted by the European Organisation for Research and Treatment of Cancer. Cardoso Borges F, van der Graaf WTA, Saesen R, Aebi S, Amariutei AE, Bekelman J, Gorlia T, Hulstaert F, Huys I, Kluetz P, Morris MJ, Patil V, Prindiville SA, Schilsky RL, Thomson A, Treweek S, Weller M, Zuidgeest M, Retel V, Lacombe D. Lancet Oncol. 2025 May;26(5):e253-e263. doi: 10.1016/S1470-2045(24)00756-3. PMID: 40318657.

7. Artificial Intelligence and Machine Learning Innovations to Improve Design and Representativeness in Oncology Clinical Trials. Azenkot T, Rivera DR, Stewart MD, Patel SP. Am Soc Clin Oncol Educ Book. 2025 Jun;45(3):e473590. doi: 10.1200/EDBK-25-473590. PMID: 40403202.

8. A Unified Approach to Health Data Exchange: A Report From the US DHHS. Abbasi AB, Layden J, Gordon W, Gregurick S, DeLew N, Grossman J, Bierman AS, Monarez S, Curtis LH, Viall AH, Rocca M, Rivera DR, Marston H, Mugge A, Smith SR, Bent K, Macrae J, Sheehy A, Wegrzyn RD, Valdez RB, Johnson C, Bush L, Blum J, Cohen MK, Bertagnolli MM, Califf RM, Tripathi M. JAMA. 2025 Jan 18. doi: 10.1001/jama.2025.0068. PMID: 39826091.

9. Summary from the NCI clinical trials planning meeting on next generation of clinical trials in non-muscle invasive bladder cancer. External Link Disclaimer. Weinstock C. Bladder Cancer. 2025;11(1). Doi:10.1177/23523735251319185.

10. Dosage Optimization of Pediatric Oncology Products: Our Patients Deserve Better. Wessel KM, Drezner N, Shord SS, Shah M, Theoret MR, Donoghue M. Clin Cancer Res. 2025 May 6. doi: 10.1158/1078-0432.CCR-24-4325. PMID: 40325515.

11. Keeping the End in Mind: Reviewing U.S. FDA Inspections of Submissions including Real-World Data. Grandinetti C, Rivera DR, Pai-Scherf L, Choe A, Kluetz PG, Kraus S, Innes GK, Ayalew K. Ther Innov Regul Sci. 2025 May 24. doi: 10.1007/s43441-025-00791-1. PMID: 40413363.

12. Beyond maximum grade: using patient-generated data to inform tolerability of treatments for haematological malignancies. Bhatnagar, Vishal et al. The Lancet Haematology, Volume 12, Issue 6, e463 - e469.

13. Beyond maximum grade: introduction to The Lancet Haematology Adverse Events Reporting Series. Thanarajasingam, Gita et al. The Lancet Haematology, Volume 12, Issue 6, e403 - e406.

14. The BCR-SEQC initiative. Mallik, R., Kusko, R., Baines, A.C. et al. Nat Immunol (2025).

15. Modernizing Research and Evidence Consensus Definitions: A Food and Drug Administration-National Institutes of Health Collaboration. Rivera DR, Cutler TL, McShane L, Meeker-O'Connell A, Marston HD, Califf RM, Bertagnolli MM; FDA-NIH MoRE Glossary Working Group; Avilés-Santa L, Ayalew K, Bayha R, Chetta J, Concato J, Culp M, Dodd L, Facey N, Forshee R, Freedman A, Friedman P, Golfinopoulos E, Grandinetti C, Jarman-Miller H, Jorgenson L, Karesh A, Kline L, Kluetz PG, Kraus S, Lee JJ, Lerro CC, Levenson M, Li J, Lu Y, Moore C, Ordóñez AE, Potter G, Prindiville S, Rosenberg Y, Sarosy G, Shapley S, Smith ER, Talley A, Tamburro R, Viviano C, Yongpravat C. JAMA Netw Open. 2025 Jun 2;8(6):e2516674. doi: 10.1001/jamanetworkopen.2025.16674. PMID: 40526378.

16. Opportunities for Pragmatic Design Elements in Surgical Trials. Abbasi A, Weltz A, Rivera DR, Nagel S, Mehta GU, Ashar BS, Cunningham SC. Opportunities for Pragmatic Design Elements in Surgical Trials. JAMA Surg. 2025 Aug 27. doi: 10.1001/jamasurg.2025.3041. PMID: 40864447.

17. Assessing tolerability of cancer therapeutics: A regulatory perspective from the FDA oncology Center of Excellence. Bhatnagar V, Lee Johnson L, Fiero MH, Mulkey F, Kluetz P. Cancer 2025. 2025 Oct 15; 131 Suppl 2(Suppl2):e35949. doi: 10.1002/cncr.35949. PMID: 41123575; PMCID: PMC12542928.

Dr. Kelly Norsworthy (OCE) with
Dr. Chenyu Lin (Duke University)

American Society of Hematology Annual Meeting, where Dr. Lin presented results from an OCE-funded research project through Triangle Center of Excellence in Regulatory Science & Innovation (CERSI): "Real-World Clinical Outcomes of IDH1 and IDH2 Inhibitors in Acute Myeloid Leukemia."

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