
# 2020

# ANNUAL REPORT

## ONCOLOGY CENTER OF EXCELLENCE

FDA U.S. FOOD & DRUG ADMINISTRATION

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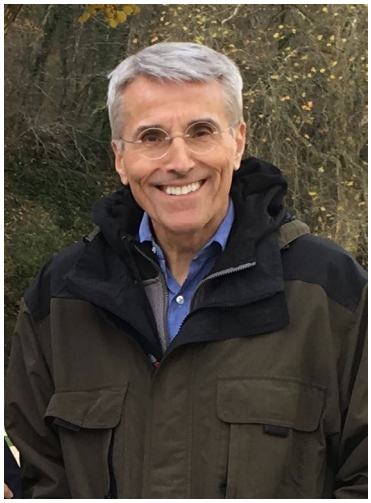
# OCE ANNUAL REPORT DIRECTOR'S MESSAGE 2020

## We Have Not Forgotten Patients With Cancer During the COVID-19 Pandemic

*January 2021*



**Richard Pazdur, MD**

As the Oncology Center for Excellence (OCE) marked its third anniversary one year ago, in January 2020, the COVID-19 pandemic began, causing world-wide upheaval in our society and the medical community. Since then, the FDA has focused tremendous effort on addressing the COVID-19 population, and rightly so. Here at the OCE, our attention has been devoted to patients with cancer to make sure they were not forgotten during this pandemic.

Our work has continued without interruption and with renewed energy to face the challenges of patients with cancer during this period of upheaval. The OCE participated in writing a guidance emphasizing the flexibility the FDA would exercise with regard to conduct of clinical trials with decentralized trial procedures aimed at patient safety. Patients often cannot go back to major cancer centers, and some of the testing and doctor visits can be conducted in communities nearer to patients' homes, so the guidance allows for that. The OCE went on to issue over a dozen guidances in 2020 on many aspects of oncology drug development.

As our staff shifted to working from home in mid-March, the work never stopped from a regulatory standpoint. Overall in 2020, there were a total of 40 new indications and 19 new molecular entities (NMEs) that were approved for oncology indications. Many approvals were aimed at reducing the interaction of patients with the health care provider to limit potential COVID exposure. These included subcutaneous dosing formulations, increasing dosing intervals, and oral formulations of already approved drugs.

Several novel drugs were approved for the treatment of mesothelioma, RET-fusion positive lung cancer, refractory myeloma, initial treatment of MSI-high colon cancer, and refractory GI stromal tumors to name a few. The OCE also worked with other divisions outside of oncology to "re-purpose" oncology drugs for potential use to combat COVID-19 infections and their sequela.

Although in-person workshops were cancelled, the OCE shifted to online listening sessions with various stakeholders, including patient groups and professional societies. More than 11 listening sessions were held from March through end of year and the OCE heard repeatedly from patients with cancer about their feeling of being forgotten during the pandemic. We hope to continue these dialogues and deepen our interactions, so that far from feeling forgotten, patients feel their voices are heard at the OCE.

**OCE at Year Four: Encouraging Diverse Voices**

In the past year, the OCE began two major initiatives, **Project Equity** and **Project Silver**, which focus on increasing minority and geriatric patient enrollment in clinical trials and bringing the voice of under-represented populations to the world of drug development. These projects join previously established OCE initiatives on diversity and inclusion, including **Project Community**, which provides outreach, education, and connection between FDA reviewers and under-represented communities in the U.S., and the **OCE Summer Scholars Program**, which provides a summer learning experience for high school students from groups under-represented in science and medicine.

The OCE also launched **Project Patient Voice**, an initiative to incorporate symptomatic side effect assessment by the patient- rather than the medical professional- to inform the tolerability of cancer therapies. This is our attempt to communicate patient-reported outcome data from clinical trials to the medical community in a way that can assist healthcare providers in discussing the side effect profile of anti-cancer treatments with their patients.

**Looking Back and Looking Forward**

In 2020, four OCE projects conducted progress assessments and published their findings:

- **Project Orbis**, the OCE's international program, reported 38 approvals in its first year working with Australia, Brazil, Canada, Singapore, Switzerland, and, most recently, the United Kingdom, to conduct collaborative reviews of important product applications. This project stemmed from our observation that many countries receive pharmaceutical applications months to years after their filing in the United States.
- **Real-Time Oncology Review** facilitates earlier submission of topline results and datasets to support an earlier start to the FDA oncology application review. This is particularly important because many of these drugs are breakthrough therapies that offer substantial improvements to patients with cancer. From February 2018 to April 2020, RTOR supported the submission and review of 20 oncology drug approvals.

- **Project Renewal**, a public health initiative OCE established to update the labeling information for older oncology products by creating a rigorous repeatable process to evaluate relevant scientific evidence from published literature, is underway.
- **Project Facilitate**, the OCE's call center to assist oncology care providers in submitting Expanded Access requests, provided data on positive trends in decreased processing times and increased number of requests in its first year, consistent with OCE's mission to improve efficiency of the Expanded Access Program and ensure equitable access to all oncology patients. However, since April 2020, the number of oncology Expanded Access requests have decreased likely due to the pandemic.

Looking forward, the OCE is engaging in **Project 2025**, an effort to envision the next five years in cancer drug development and leverage our resources and talents to improve collaboration with stakeholders to move the field forward as quickly as possible.

I hope you enjoy learning more about the OCE's work described in this report and welcome your involvement and interest in the coming year to advance our mission.

**Richard Pazdur, MD**

*Director, Oncology Center of Excellence*

# OCE ORGANIZATIONAL STRUCTURE

## OUR MISSION:

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

## OUR VISION:

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

## OCE KEY STAFF:

**Center Director:**
Richard Pazdur, MD

**Deputy Center Director:**
Paul G. Kluetz, MD

**Deputy Center Director:**
Marc R. Theoret, MD

**Chief of Medical Oncology:**
Julia A. Beaver, MD

**Director for Regulatory Affairs and Regulatory Policy:**
Tamy Kim, PharmD

**Associate Director (AD) for Pediatric Oncology:**
Gregory Reaman, MD

**AD for Oncology Devices (Acting) (also Chief Medical Officer, Office of Surgical and Infection Control Devices, CDRH):**
Dorian M. Korz, MD

**AD for Oncology In vitro Diagnostics (also Director of Personalized Medicine, CDRH):**
Wendy Rubinstein, MD, PhD

**AD for Cell and Gene Therapy:**
Adnan Jaigirdar, MD

**AD for Tissue Agnostic Drug Development:**
Steven Lemery, MD

**AD for Strategy and Partnerships:**
Julie Schneider, PhD

**AD for Global Regulatory Outreach:**
Dianne Spillman, BS

**AD for Communications:**
Kirsten B. Goldberg, MA

**AD for External Outreach and Engagement:**
Rea Blakey, BS

**AD for Education:**
Jennifer Gao, MD

**AD for Patient Outcomes:**
Vishal Bhatnagar, MD

**AD for Oncology in Older Adults and Special Populations (Acting):**
Harpreet Singh, MD

**AD for Global Clinical Sciences (Acting):**
R. Angelo de Claro, MD

**AD for Science & Policy to Address Disparities (Acting):**
Lola Fashoyin-Aje, MD, MPH

**AD for Safety (Acting):**
Meredith Chuk, MD

**AD for Oncology Labeling (Acting):**
CAPT William Pierce, PharmD, MPH, BCPS

**AD for Pharmacoepidemiology:**
Donna R. Rivera, PharmD., MSc.

**Director for Oncology Program Operations:**
Sherwin Sapasap, MS, MBA, MHA

# REGULATORY REVIEW

Authorized by the 21st Century Cures Act, the OCE was established in January 2017 to facilitate the development and clinical review of oncology products by uniting scientific experts across the FDA's product centers to conduct expedited review of drugs, biologics, and devices.

## 2020 ONCOLOGY APPROVALS*

| ONCOLOGY APPROVALS | |
|---|---|
| NMEs/ Original BLA | 19 (8 Regular, 11 AA) |
| Supplements (new indication) | 40 (32 Regular, 8 AA) |
| Supplements (new population) | 2 (2 Regular, 0 AA) |
| 505b2 | 6 |
| Biosimilar | 1 |
| PMA - Original | 7 |
| PMA - Supplemental | 12 |

## 2020 SUBMISSIONS REVIEWED UNDER EXPEDITED PROGRAMS*

| SUBMISSIONS REVIEWED UNDER EXPEDITED PROGRAMS | GRANTED |
|---|---|
| Breakthrough Designation | 28 |
| Breakthrough Device Designation | 9 |
| Regenerative Medicine Advanced Therapy | 6 |
| Fast Track | 88 |
| Priority Review | 78 |

* Approval numbers reflect approvals from CBER, CDER and CDRH

# HIGH IMPACT APPROVALS IN 2020

| PRODUCT | INDICATION | ADDITIONAL INFORMATION |
|---|---|---|
| SARCLISA (isatuximab-irfc) | SARCLISA (isatuximab-irfc) in combination with pomalidomide and dexamethasone (IsaPd) is proposed for the treatment of patients with multiple myeloma (MM) who have received at least two prior therapies, including lenalidomide and a proteasome inhibitor (PI). | Link |
| Koselugo (selumetinib) | KOSELUGO (selumetinib) is indicated for the treatment of pediatric patients 2 years of age and older with neurofibromatosis type 1 (NF1) who have symptomatic, inoperable plexiform neurofibromas (PN). | Link |
| Jelmyto (mitomycin) | Jelmyto is an alkylating drug indicated for the treatment of adult patients with low-grade Upper Tract Urothelial Cancer (LG-UTUC). | Link |
| Pemazyre (pemigatinib) | PEMAZYRE (pemigatinib) is indicated for the treatment of adults with previously treated, unresectable locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or other rearrangement as detected by an FDA-approved test. | Link |
| Tukysa (tucatinib) | TUKYSA (tucatinib) is a kinase inhibitor drug indicated in combination with trastuzumab and capecitabine, for adult patients with advanced unresectable or metastatic HER2-positive breast cancer, including patients with brain metastases, who have received one or more prior anti-HER2-based regimens in the metastatic setting. | Link |
| Trodelvy (sacituzumab govitecan-hziy) | TRODELVY (sacituzumab govitecan-hziy) is indicated for the treatment of adult patients with metastatic triple-negative breast cancer (mTNBC) who have received at least two prior therapies for metastatic disease. | Link |
| Tabrecta (capmatinib) | TABRECTA (capmatinib) for the treatment of adult patients with metastatic non-small cell lung cancer (NSCLC) whose tumors have a mutation that leads to mesenchymal-epithelial transition (MET) exon 14 skipping as detected by an FDA-approved test. | Link |
| Retevmo (selpercatinib) | RETEVMO (selpercatinib) is indciated for:<br>Adult patients with metastatic RET fusion-positive non-small cell lung cancer (NSCLC);<br>Adult and pediatric patients 12 years of age and older with advanced or metastatic RET-mutant medullary thyroid cancer (MTC) who require systemic therapy;<br>Adult and pediatric patients 12 years of age and older with advanced or metastatic RET fusion-positive thyroid cancer who require systemic therapy and who are radioactive iodine-refractory (if radioactive iodine is appropriate). | Link |
| KEYTRUDA (pembrolizumab) | KEYTRUDA (pembrolizumab) is indicated for the treatment of adult and pediatric patients with unresectable or metastatic tumor mutational burden high (TMB H) [≥10 mutations/megabase (mut/Mb)] solid tumors, as determined by an FDA-approved test, that have progressed following prior treatment and who have no satisfactory alternative treatment options. This approval includes the following Limitations of Use: the safety and effectiveness of KEYTRUDA in pediatric patients with TMB H central nervous system cancers have not been established. | Link |
| INQOVI (decitabine and cedazuridine) | INQOVI (decitabine and cedazuridine) is indicated for the treatment of adult patients with myelodysplastic syndromes (MDS), including previously treated and untreated, de novo and secondary MDS with the following French-American-British subtypes (refractory anemia, refractory anemia with ringed sideroblasts, refractory anemia with excess blasts, and chronic myelomonocytic leukemia [CMML]) and intermediate-1, intermediate-2, and high-risk International Prognostic Scoring System groups. | Link |
| Onureg (azacitidine) | ONUREG (azacitidine) is a nucleoside metabolic inhibitor indicated for continued treatment of adult patients with acute myeloid leukemia who achieved first complete remission (CR) or complete remission with incomplete blood count recovery (CRi) following intensive induction chemotherapy and are not able to complete intensive curative therapy. | Link |
| Opdivo (nivolumab) | OPDIVO (nivolumab), in combination with ipilimumab, for the treatment of adult patients with unresectable malignant pleural mesothelioma. | Link |

## COVID-19 AND ONCOLOGY PRODUCT APPROVALS

The ongoing COVID-19 pandemic has affected everyone, with an immense impact on current and future patients with cancer. The OCE staff has provided input on multiple guidance documents, including the "Conduct of Clinical Trials of Medicinal Products during COVID-19 Public Health Emergency" guidance and initiated the "Institutional Review Board (IRB) Review of Individual Patient Expanded Access Requests for Investigational Drugs and Biological Products During the COVID-19 Public Health Emergency" guidance. The FDA oncology review teams have also maintained their scientific rigor in reviewing applications and implementing flexibilities where appropriate. In 2020, the Office of Oncologic Diseases approved 18 NMEs, and 40 new indications for the treatment of patients with cancer (1 NME was approved in CBER for a total of 19 NME approvals in cancer for 2020). Several approvals were aimed at reducing the interaction of patients with the health care provider to limit potential COVID-19 exposure. These included subcutaneous dosing formulations, longer dosing intervals, and oral formulations of already approved drugs. The OCE remains committed to advancing treatment options for patients with cancer, including those that can help minimize exposure, while continuously striving to fulfill its mission of achieving patient-centered regulatory decision-making through innovation and collaboration. Additional information on FDA oncology approvals with COVID-19 implications can be found here.

## REAL-TIME ONCOLOGY REVIEW (RTOR)

The Real-time Oncology Review (RTOR) program, initiated in 2018, permits the FDA to access topline results and datasets, after datasets are locked, to support an earlier start to the application review. Initially, RTOR was created to support supplemental drug applications and was later expanded to include new molecular entities (NMEs). RTOR has enabled the approval of some applications only a few weeks following formal submission. In 2020, the OCE approved 3 NME/Original BLA applications and 13 supplemental applications using the RTOR program.

## ASSESSMENT AID (AAID)

The Assessment Aid (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 the FDA's review and assessments to focus on critical thinking regarding the adequacy of the data and strength of results and reduces the amount of time spent on recapitulating information and administrative tasks, such as formatting. In 2020, the OCE approved 12 NME applications and 31 supplemental applications using the Assessment Aid.

## PROJECT ORBIS

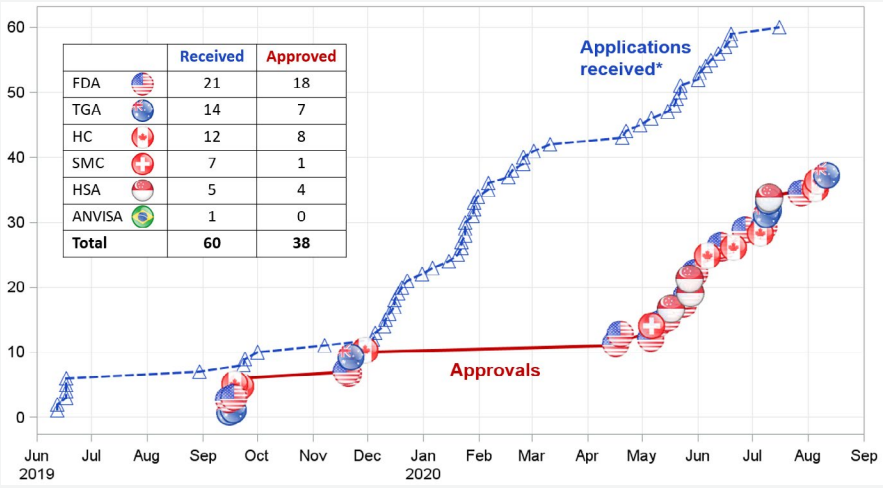
Project Orbis is the global collaborative review program begun by OCE in May 2019 to expedite access to innovative cancer therapies across multiple countries through coordination and submission of oncology marketing applications to the respective regulatory authorities. Current Project Orbis partners (POP) include the regulatory health authorities of Australia (May 2019), Canada (May 2019), Switzerland (December 2019), Singapore (December 2019), Brazil (May 2020), and the United Kingdom (January 2021). Each participating country retains full independence in regulatory decision making and labeling negotiations. FDA coordinates quarterly meetings with the POP to discuss scientific, regulatory, and operational aspects of the program.

In the first year of Project Orbis (June 2019 to June 2020), a total of 60 oncology marketing applications were received across the countries, representing 16 unique projects, and resulting in 38 approvals. New molecular entities, also known as new active moieties, comprised 28% of the received marketing applications. The median time gap between FDA and Orbis submission was 0.6 months. The median time-to-approval was similar between FDA (4.2 months) and the POP (4.4 months).

Looking ahead to 2025, FDA together with the other Orbis countries plan to continue and expand collaborative efforts between regulatory authorities to support global oncology drug development and regulatory review.

**FIGURE 1:**
Project Orbis Marketing Application Submissions and Approvals: Year One Experience.

*Initial set of Orbis applications based on 21 FDA applications received from 12 Jun 2019 to 12 Jun 2020. Abbreviations: FDA, Food and Drug Administration (USA); TGA, Therapeutic Goods Administration (Australia); HC, Health Canada; SMC, Swiss Agency for Therapeutic Products (Swissmedic) (Switzerland); HSA, Health Sciences Authority (Singapore); ANVISA, Brazilian Health Regulatory Authority.



# OCE PROGRAMS

## IMMUNO-ONCOLOGY

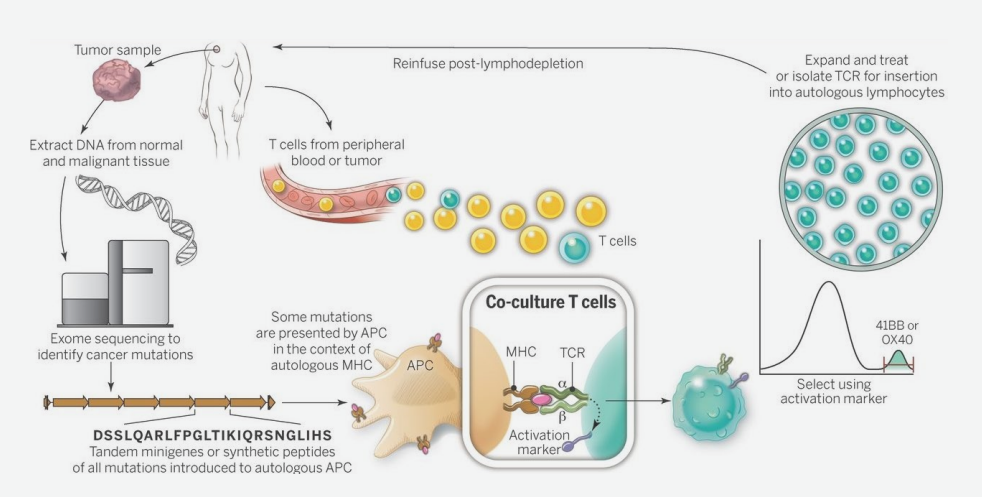
Immuno-oncology (I-O) therapeutics is leading to a transformational shift in treatment paradigms for patients with cancer. The OCE Immuno-Oncology Therapeutics Program (IOTP) brings together existing expertise across the FDA and promotes development of new therapeutics that harness the immune system to engage new, more efficacious treatment paradigms for patients with cancer. In 2020, along with 16 approvals of immune checkpoint inhibitors for new indications across several cancer types, rapid approval of an extended dosing regimen to reduce the frequency that patients needed to present the clinic for infusions, the second tissue agnostic indication was approved for patients with cancer harboring a high tumor mutation burden. The I-O therapeutics program is supporting new regulatory science research for I-O drug development, internally and externally, including funding opportunities for I-O research through the Broad Agency Announcement. Programs were established for internal staff development in I-O drug development. Lastly, the I-O program has supported multiple stakeholder engagements to address the challenges and opportunities for development of I-O drugs as combination regimens and in patients with resistance to prior cancer immunotherapy.

## ONCOLOGY CELL AND GENE THERAPY

The Oncology Cell and Gene Therapy program focuses on clinical evaluations for, and helps to expedite development of, cellular cancer therapies. Examples include T-cells modified with chimeric antigen receptors (CAR Ts) or with T-cell receptors with redirected specificity (TCR-Ts), and developed using technologies including gene-editing, e.g., clustered regularly interspaced short palindromic repeats (CRISPR) or transcription activator-like effector nucleases (TALEN); novel strategies in hematopoietic stem cell transplantation (HSCT); dendritic cells; adoptive T-cell therapies; tumor neoantigen-based personalized medicine (vaccine or cell therapy); natural killer cells; oncolytic bacteria and viruses; therapeutic cancer vaccines; therapies that modulate the microbiome; and combinations of these therapeutics with hematopoietic stem cell transplantation, checkpoint inhibitors, chemotherapies, radiation and other agents.

Using the OCE's Medical Oncology Review and Evaluation (MORE) team that includes a medical oncology specialist and specialists from the FDA's Center for Biologics Evaluation and Research (CBER), OCE has leveraged the combined skills of the FDA clinical oncology review staff to conduct the clinical review for CBER's latest approval of the CAR T therapy in 2020, TECARTUS (brexucabtagene autoleucel), the first cell-based gene therapy for the treatment of relapsed or refractory mantle cell lymphoma (MCL).

To facilitate rapid research and development so that more patients with cancer can benefit from cell therapy, OCE has funded an extramural, cutting-edge research project on cell therapy through FDA's Advancing Regulatory Science Broad Agency Announcement (BAA).




## ONCOLOGY DEVICES AND DIAGNOSTICS

Oncology devices and diagnostics are reviewed and regulated by the Center for Devices and Radiological Health (CDRH) in partnership with OCE. In 2020, CDRH and OCE worked toward our common goal of promoting innovation, patient-centered oncologic approaches, and education.

Promoting innovation has always been a priority of CDRH. 2020 has seen new oncology device studies seeking FDA oversight for indications in many cancer areas, some notable examples include: liver cancer and metastases, lymph node mapping, differentiation of malignancies from healthy tissues and lymph nodes, novel arterial infusion methods, and treatment of cancer therapy side-effects such as radiation dermatitis and lymphedema. We continue to foster advancement with our Breakthrough Devices Program for certain medical devices and device-led combination products that provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating diseases or conditions. CDRH has granted breakthrough status to several devices for oncological indications, including those for the detection of skin lesions and cancer.

CDRH cleared the Stryker endoscopic fluorescence imaging device for use in intraoperative lymphatic mapping and tissue perfusion. The Merit Medical System was cleared for obtaining biopsies from soft tissues and lymph nodes. The Everlift Submucosal Lifting Agent is indicated for use in gastrointestinal endoscopic procedures for

submucosal lift of polyps, adenomas, early-stage cancers, or other gastrointestinal lesions prior to excision with a snare or other appropriate endoscopic device. HANAROSTENT, an esophageal stent, provides palliative relief for cancer patients by maintaining esophageal luminal patency in esophageal strictures caused by intrinsic and/or extrinsic malignant tumors.

Continuing our role of educating patients and health care providers about the benefits and risks of medical devices, CDRH updated the FDA website for medical device reports of breast implant-associated anaplastic large cell lymphoma (BIA-ALCL), a type of non-Hodgkin's lymphoma and a known risk from breast implants. Additionally, to inform and engage stakeholders, we presented Innovations in Cancer Treatment Devices at the 2020 Generis American Medical Device Summit.

In 2020, FDA authorized numerous in vitro diagnostic devices for oncology indications, including thirteen contemporaneous new companion diagnostic approvals. Seven companion diagnostics were approved for use in in patients with non-small cell lung cancer (NSCLC) to guide treatment with a total of nine therapeutic agents. Targeted treatment of cholangiocarcinoma, a less common malignancy, was for the first time supported by a tissue-based companion diagnostic which detects FGFR2 fusions and rearrangements (FoundationOne CDx). Another first indication for a companion diagnostic was for follicular lymphoma (cobas EZH2 Mutation Test, Roche Molecular Systems, Inc.).

The first two next-generation sequencing (NGS) - based tumor agnostic companion diagnostic indications were approved in 2020, both for adult and pediatric patients with solid tumors. The first indication is for the identification of patients with unresectable or metastatic tumor mutational burden-high (TMB-H) tumors for consideration of immune checkpoint inhibitor therapy. The second is for the identification of gene fusions in NTRK1, NTRK2, and NTRK3 for targeted therapy. Both tumor agnostic indications were approved for the FoundationOne CDx assay (Foundation Medicine, Inc.).

The first two NGS-based liquid biopsy companion diagnostics were approved in 2020: Guardant360 CDx, a 55 gene assay manufactured by Guardant Health and FoundationOne Liquid CDx, a 311 gene assay

manufactured by Foundation Medicine, Inc. These tests enable less invasive testing to be used and facilitate clinical assessment in cases where standard tissue biopsies are not feasible. Both of these diagnostics can provide biomarker information for solid tumors, and the companion diagnostic indications provide a basis for selecting targeted therapies for several advanced malignancies. Both have companion diagnostic claims for evaluation of specific EGFR mutations in metastatic NSCLC. In addition, the FoundationOne Liquid CDx assesses patients with NSCLC for ALK-targeted therapy and can be used for treatment selection in patients with metastatic castrate-resistant prostate cancer (BRCA1, BRCA2, ATM), breast cancer (PIK3CA), and ovarian cancer (BRCA1, BRCA2). If the specific mutations or alterations associated with companion diagnostic claims are not detected by these liquid biopsy tests, then a tumor biopsy should be performed to determine if the specific mutations or alterations are present in the patient's tumor.

For a complete list of FDA-cleared or approved companion diagnostic devices, see: List of Cleared or Approved Companion Diagnostic Devices (In Vitro and Imaging Tools). This page has expanded from specific therapeutic products to also include specific groups of oncology therapeutic products in accordance with guidance published April, 2020, "Developing and Labeling In vitro Companion Diagnostic Devices for a Specific Group of Oncology Therapeutic Products". This guidance will facilitate the development and use of more than one companion diagnostic test that is essential for the safe and effective use of a corresponding group of personalized medicine cancer therapies. By helping device developers bring companion diagnostics with broader labeling claims to market, FDA intends to optimize patient care by providing health care providers with greater flexibility in choosing the most appropriate treatment for patients with cancer.

## ONCOLOGY REGULATORY AFFAIRS AND POLICY

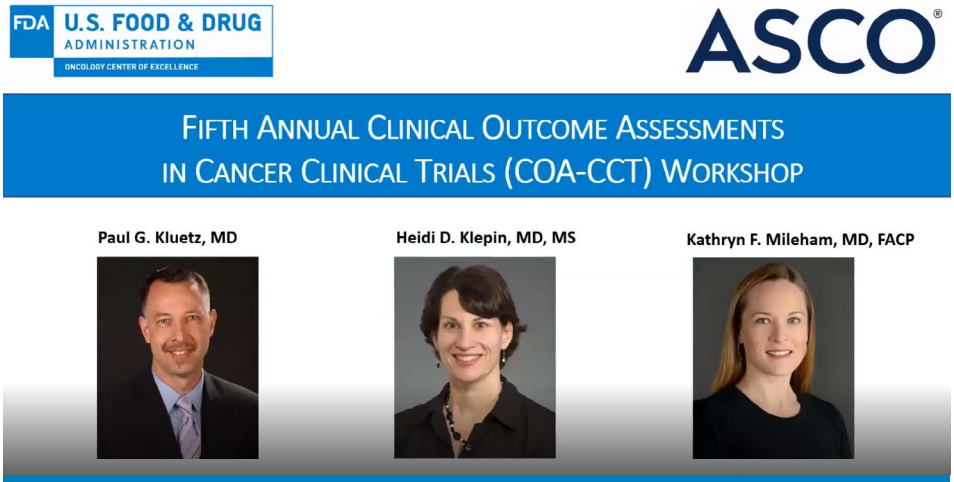
The OCE Oncology Regulatory Affairs and Policy program provides guidance on complex regulatory issues to the review divisions as they interact with sponsors, and to external stakeholders. This program works with each of the OCE Programs to implement processes across multi-disciplinary review teams. To date, this program has developed and/or implemented processes and programs for Project Facilitate, Project Renewal, Project Orbis, OCE MORE Teams, RTOR, Assessment Aid, OCE Pediatric Review Committee (PeRC), and the review of applications for products intended to treat COVID-19 in patients with cancer. The Oncology Regulatory Affairs and Policy program is generally involved in some manner in most OCE programs to provide guidance from a regulatory standpoint or to manage the program.

The Oncology Regulatory Affairs and Policy program also manages Project Facilitate, a first of its kind national program and call center to manage and conduct the clinical review of expanded access requests for investigational drugs for patients with cancer.

## PATIENT-FOCUSED DRUG DEVELOPMENT

The OCE Patient-Focused Drug Development (PFDD) program fosters collaboration between FDA Centers and external stakeholders involved in patient outcomes research in cancer populations. In 2020, the PFDD program launched Project Patient Voice, a web-based platform to communicate patient-reported symptomatic side effects to health care providers and patients. (See page 21 for details about this exciting initiative.) Additional areas where the PFDD program advanced its mission in 2020 include:

- **GENERATING SCIENCE-BASED RECOMMENDATIONS.** The PFDD program assisted FDA Centers and review divisions by providing scientific advice regarding use of patient-reported outcomes (PRO) and other clinical outcome assessments in over 30 sponsor meetings and the review of 22 cancer product applications.
- **ENGAGING WITH PATIENTS AND ADVOCACY GROUPS**. The PFDD program held its fifth annual Clinical Outcomes Assessments in Cancer Clinical Trials workshop on July 17, 2020. The workshop was co-sponsored with the American Society of Clinical Oncology (ASCO) and brought over 1,000 patients and drug development stakeholders together to discuss assessment of patient-reported symptomatic side effects and introduced the launch of Project Patient Voice.
- **FOSTERING RESEARCH INTO MEASUREMENT OF SYMPTOMS AND FUNCTION.** The PFDD program funded two external collaborations in 2020 to advance measurement of physical function in patients with cancer using PRO, wearable devices and other sources. The program also generated its own in-house patient-focused research, supporting 3 ORISE fellows and producing over 20 peer-reviewed manuscripts and abstracts this year. In addition, the program expanded its international and cross-governmental research consortia on meaningful research to advance measurement of symptoms and function.




OCE Deputy Director Paul Kluetz, MD, and ASCO Chairs Heidi Klepin, MD and Kathryn Mileham, MD, opening the 2020 Clinical Outcomes Assessment in Cancer Clinical Trials workshop.

## PEDIATRIC ONCOLOGY

The Pediatric Oncology Program provides the OCE with the focused expertise in cancer drug development for childhood cancer leveraging both drug discovery for the common cancers in adults and the specific legislative initiatives surrounding drug development and approval for children.

Facilitating early pediatric studies of appropriate new, targeted cancer therapeutics and eliminating the 6 1/2 year time lag between first in human and first in children studies of approved cancer drugs is the first step in achieving 2025 goals to accelerate pediatric cancer drug development.

This regulatory landscape change has resulted from the full implementation of Sec. 504 of FDARA and the amendments to PREA made possible by the RACE for Children Act. Although not the solution to all of the challenges to drug development in a rare disease population, a fundamental obstacle to timely study, potential approval, and earlier access to new drugs has been addressed.

Successful implementation is supported by several initiatives:

- Formation of the Oncology Subcommittee of the Pediatric Review Committee (PeRC) to review Pediatric Study Plans (PSPs) and Proposed Pediatric Study Requests (PPSRs) and Written Requests (WR). During weekly meetings OCE PeRC has reviewed:

**June 17-18, 2020 pedsODAC Meeting Roster**

| pedsODAC DFO *(Acting)* | ONCOLOGIC DRUGS ADVISORY COMMITTEE MEMBERS | | |
|---|---|---|---|
|  LaToya Bonner, PharmD *(FDA, Non-Voting)* |  David Mitchell *(Consumer Rep.)* |  Alberto Pappo, MD *(Chairperson)* |  Jonathan D. Cheng, MD *(Industry Rep., Non-Voting)* |
| **TEMPORARY MEMBERS** | | | |
|  Catherine Bollard, MBChB, MD |  Steven G. DuBois, MD |  Ira J. Dunkel, MD |  Julia Glade Bender, MD |
|  Richard Gorlick, MD |  Katherine A. Janeway, MD, MMSc |  Naynesh R. Kamani, MD |  E. Anders Kolb, MD |
|  Theodore W. Laetsch, MD |  Donna Ludwinski *(Patient Rep.)* |  Tobey J. MacDonald, MD |  Leo Mascarenhas, MD, MS |
|  D. Williams (Will) Parsons, MD, PhD |  Elizabeth Raetz, MD |  Nita Seibel, MD |  Malcolm A. Smith, MD, PhD |
| **FDA PARTICIPANTS *(Non-Voting)*** | | | |
|  Denise Casey, MD |  Leslie Doros, MD |  Gregory Reaman, MD |  Megan Zimmerman, MD |


Gregory Reaman, MD

| PEDIATRIC ONCOLOGY PROGRAM REVIEWS FOR 2020 | |
|---|---|
| iPSPs | 154 |
| Agreed iPSPs | 96 |
| Amended Agreed iPSPs | 19 |
| PPSRs | 14 |
| WRs | 9 |
| Amended WRs | 7 |
| Type F Meetings | 15 |

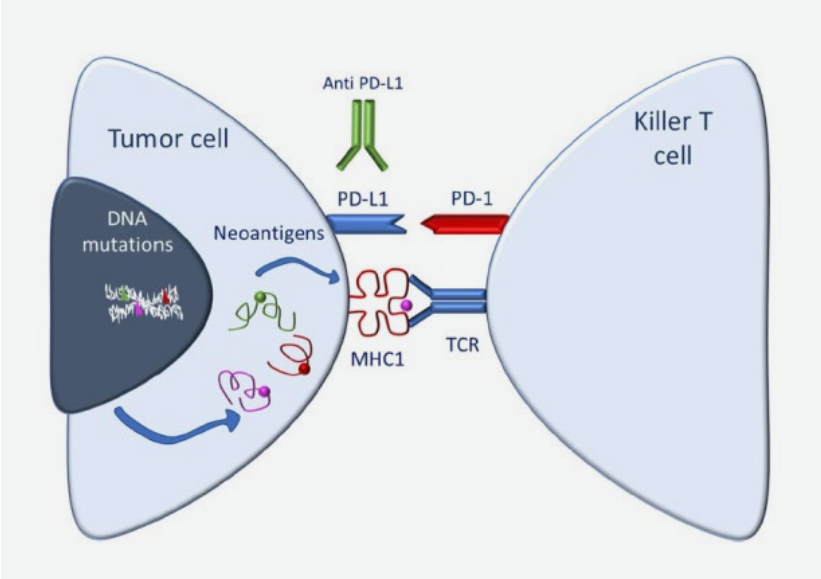
- Active participation in Pediatric Liaison meetings with the trade organizations BIO and PhRMA by Dr. Gregory Reaman and Pam Balcazar of the OCE.
- ASCO and AACR Educational sessions; active representation/participation in the AACR Pediatric Cancer Working Group
- Participation in webinars/workshops of the pediatric cancer advocacy groups: Children's Cause for Cancer Advocacy, the Coalition for Childhood Cancer, and CureSearch for Childhood Cancer
- Active participation in the Steering Committee of ACCELERATE, an international, multi-stakeholder platform to foster coordination and collaboration for global pediatric cancer drug development
- Planning and participation in the 7th Annual ACCELERATE Pediatric Oncology Conference
- Planning and participation in ACCELERATE Pediatric Strategy Forums: 1) New Therapeutics for Children with AML and 2) Role of epigenetic modifiers in the treatment of pediatric cancers
- Active participation in the Steering Committee of the NCI's Childhood Cancer Data Initiative (CCDI) and the National Childhood Cancer Registry to aid Real World Data and Evidence strategies to advance childhood cancer drug development
- Active participation in the design team and Steering Committee and development of the FNIH public-private partnership, ACT4Kids (Advancing Cancer Therapies for Children) to expand pre-clinical testing of novel agents in pediatric-specific tumor models to guide drug development.

## PRECISION ONCOLOGY

Precision oncology is a medical model that individualizes and customizes healthcare delivery, medical decision making, and treatment selection. More precise targeting of a treatment to an individual's genomic, proteomic, or metabolomic profile may result in more effective and less toxic anti-cancer therapies.

Highlights of the Precision Oncology Program in 2020 included OCE involvement in multiple workshops including Detecting Circulating Tumor DNA for Cancer Screening and publication of a final guidance to industry on Developing and Labeling In vitro Companion Diagnostic Devices for a Specific Group or Class of Oncology Therapeutic Products. In addition, a number of notable precision oncology approvals took place in 2020, including biomarker selected approvals for patients with metastatic non-small cell lung cancer (NSCLC) that has mesenchymal-epithelial transition exon 14 skipping mutations, NSCLC and medullary thyroid cancers with RET alterations, prostate cancer with BRCA mutations or genomic instability, and a tissue agnostic approval for patients with solid tumors with tumor mutation burden-high. There were also approvals for the first liquid biopsy next-generation sequencing-based tests.
OCE also led an education program across the centers on omics-driven oncology and biomarkers.

Looking ahead toward 2025, the Precision Oncology Program hopes to ensure and expand on development of biomarker-based clinical trial designs, including advancing policy on tissue-agnostic drug development, circulating tumor DNA, and novel imaging modalities. The program will also work with patient advocacy groups to ensure language around genomics and precision oncology is patient-friendly and easy to understand.



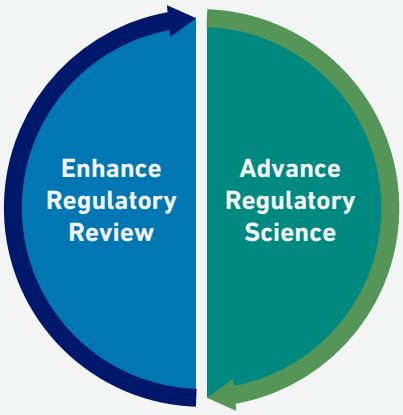

Galuppini et al. Cancer Cell Int (2019) 19:209
https://doi.org/10.1186/s12935-019-0929-4

## ONCOLOGY REAL WORLD EVIDENCE (RWE) PROGRAM

Increasing availability of real world data (RWD) presents an opportunity to obtain data from sources outside of traditional clinical trials. However, RWD is not real world evidence (RWE) without a clear research objective, appropriate methods, and data that are fit for purpose. In 2020, the OCE established an oncology specific RWE Program to advance science and policy initiatives aimed at transforming RWD into RWE that can inform regulatory decision making.

### OCE RWE PROGRAM FOCUS AREAS

| REGULATORY REVIEW | REGULATORY SCIENCE & COLLABORATION | REGULATORY POLICY | ENGAGEMENT & EDUCATION |
|---|---|---|---|




### Regulatory Review: TEAM FoRWD

To advance the regulatory use of RWE, we have assembled TEAM FoRWD (Translational Evaluation and Assessment of Methods to Facilitate use of Oncology RWD). TEAM FoRWD comprises FDA scientists with expertise in pharmacoepidemiology, hematology and oncology, epidemiology, biostatistics, and regulatory science to evaluate opportunities for RWD to generate RWE that can complement our understanding of medication risks and benefits for patients.

### RWE Research Collaborations

The Oncology RWE Program has a portfolio of collaborative research spanning cancer types and therapeutic classes evaluating clinically meaningful questions on topics including analytic methods, rwEndpoints, patient generated health data, and drug safety. Regulatory science research in RWE is linked to OCE scientific priorities in precision medicine, patient focused drug development, immuno-oncology, drug safety, health equity and special populations with the goal of fostering effective science-based regulatory review and policy.


**Oncology Real World Data (RWD) Roundtable:**
*Lessons learned from the Duke Margolis Center for Health Policy and Friends of Cancer Research*

# OCE INITIATIVES

## PROJECT FACILITATE

Project Facilitate is a program and call center to assist oncology healthcare providers and regulatory professionals in requesting access to investigational therapies for patients with cancer. It is a single point of contact where FDA oncology staff will help oncologists and their healthcare team through the process to submit an Expanded Access request for an individual patient with cancer.

Project Facilitate will be entering its second year starting 2021. Since its implementation until the end of 2020, Project Facilitate has received 1035 telephone calls and 1197 expanded access requests for individual patients. Since June 2020, Project Facilitate also started to conduct the clinical reviews for expanded access requests and has reviewed 409 expanded access requests (between June and December 2020) for individual patients with cancer with a median turnaround time of less than a day.

This program also conducts outreach with external stakeholders such as cancer centers, academia, community hospitals and patient groups in an effort to make the community aware of the option of seeking expanded access when there are no satisfactory alternatives.

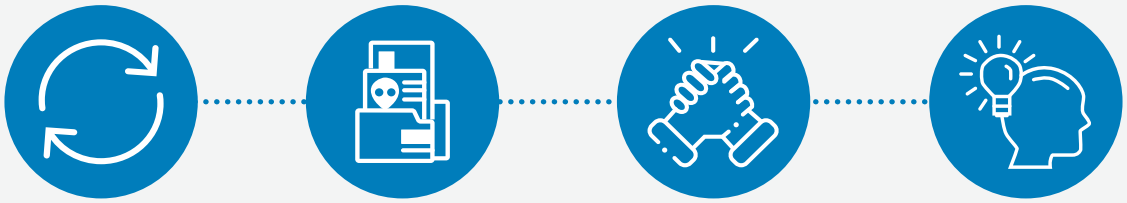

**OCE's Project Facilitate call center assists oncology healthcare providers in requesting access to investigational therapies for patients with cancer.**

## PROJECT RENEWAL

Project Renewal is an ongoing collaborative public health initiative aimed at updating the prescribing information for important long-standing oncology products. Launched in 2018 with a series of 6 workshops, Project Renewal established a set of repeatable processes to evaluate the public literature for scientific evidence to inform regulatory decisions for updating oncology product labels.

On June 23, 2020, members of the OCE leadership presented at the American Association for Cancer Research (AACR) Virtual Annual Meeting II in a session titled "Engaging the Oncology Community to Advanced Regulatory Science – FDA's Project Renewal and Project Socrates." With academic oncologists and pharmacologists as invited panelists, the discussions focused around the evolution of FDA product labeling, the key elements required for substantial evidence of effectiveness, and the educational goals of Project Renewal for the hematology and oncology fellows who participate as research team members. The session was featured as a guest post in the AACR Cancer Research Catalyst on July 28, 2020. In addition, a manuscript discussing Project Renewal processes and the ongoing external engagement and educational initiatives, titled "FDA Oncology Center of Excellence Project Renewal: Engaging the Oncology Community to Update Product Labeling for Older Cancer Drugs," has been published.

Looking ahead to 2025, the OCE looks forward to updating the labels of additional older oncology products and continuing to strengthen its ties with the oncology community.



**Develop Repeatable Processes** to evaluate scientific evidence and determine whether labeling updates are needed

**Use Published Data** to research off-label uses and develop a method to update existing labels

**Engage with Oncology Community** to increase transparency of FDA processes and encourage collaboration

**Foster Educational Experiences** for clinical fellows to learn about FDA's mission and regulatory processes

## PROJECT ACCELERATE

OCE launched Project Accelerate in February 2020 to track the oncology approvals using the accelerated approval pathway. The team created a tracking database with the accelerated approval application information and corresponding postmarketing requirement(s) to verify clinical benefit. The team also met with international regulatory authorities to discuss experience with equivalent programs such as conditional marketing authorisation program (European Union), notice of compliance with conditions (Canada), and provisional approval (Australia).

## PROJECT POINT/COUNTERPOINT

Project Point/Counterpoint pilots a new version of the Oncologic Drugs Advisory Committee (ODAC) briefing document that combines the company's and FDA's positions in one document, similar to the Assessment Aid. This document increases the transparency of differences in viewpoints and is more concise to focus on salient data and facilitate the committee's understanding of the critical issues for discussion.

The new briefing document was initially used for the December 17-18, 2019 ODAC and was favorably received by the committee members. An updated template was subsequently used to create the briefing document for the July 14, 2020, ODAC meeting.

## PROJECT PROTECT

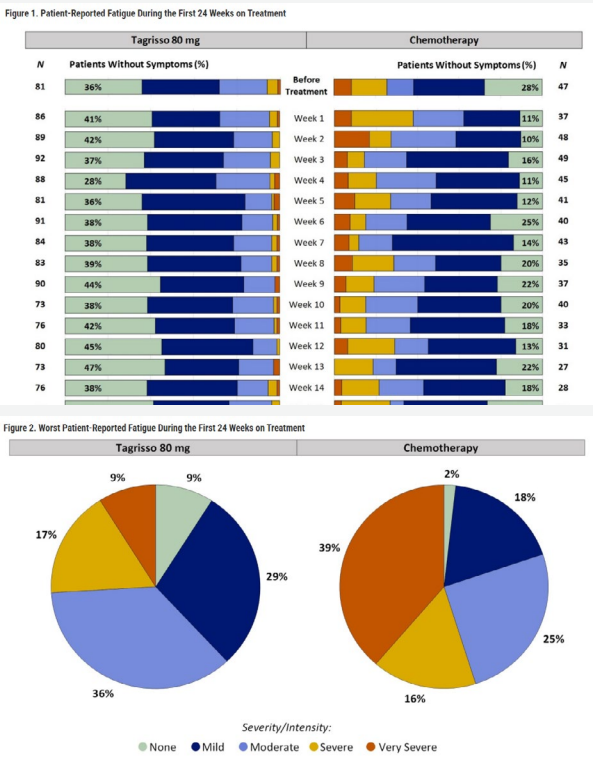
Project Protect encompasses the OCE's efforts to ensure the safety of drugs approved to treat patients with cancer. A centralized safety team works with the review divisions to provide consistent monitoring and communication of risk information across drugs within the same class throughout the lifecycle of drug development. The team has also developed technical specifications for applicants and automated review tools for data analysts and reviewers to standardize and expedite the review of safety data in NDA and BLA submissions.

## PROJECT PATIENT VOICE

On June 23, 2020, the OCE PFDD program launched Project Patient Voice to create a consistent source of publicly available information describing patient-reported symptoms from cancer trials for marketed treatments. While this patient-reported data has historically been analyzed by the FDA during the drug approval process, it is rarely included in product labeling and is largely inaccessible to the public.

Patient-reported outcome (PRO) data is collected using questionnaires that patients complete during clinical trials. The questionnaires can assess symptoms and function as well as other aspects of health-related quality of life. Patient generated information characterizing symptomatic side effects can be obtained, including how severe or how often a symptom or side effect occurs.

Making PRO data available can provide additional, complementary information for health care professionals to discuss with patients, especially when discussing the possible side effects of a cancer treatment. In contrast to standard clinician-reported safety data found in product labeling, symptom information on this website is collected directly from patients and can show symptoms before treatment starts and at multiple time points while receiving cancer treatment.




Example visualizations from the Project Patient Voice website

Project Patient Voice is in the pilot phase, and future versions of the website may include data from additional clinical trials, as well as other types of patient-reported data such as physical function or ability to carry out daily activities. The OCE received feedback on Project Patient Voice during the July 17, 2020, Clinical Outcomes Assessments in Cancer Clinical Trials workshop, as well as directly via the Project Patient Voice website to ensure that the information is clear and meaningful to health care professionals and patients.

## PROJECT SignifiCanT

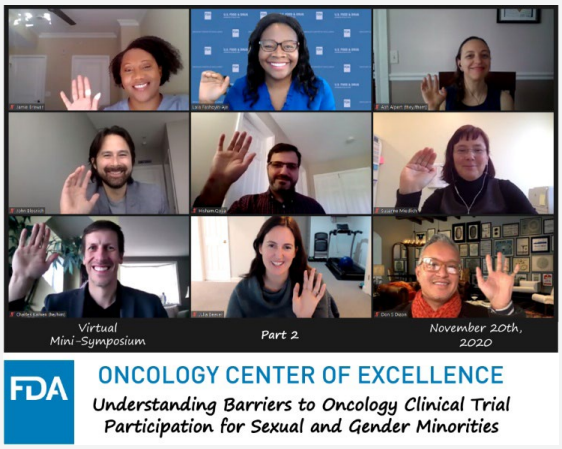
OCE's Project **S**ignifi**CanT** (**S**tatistics in **Can**cer **T**rials) was launched in 2020. The aim of this project is to promote collaboration and engagement among different stakeholders in design and analysis of cancer clinical trials to advance drug development. The objectives of the Project **S**ignifi**CanT** are to provide a platform to promote non-product specific scientific discussions on clinical and statistical aspects of design and analysis of cancer clinical trials, and foster collaboration among regulators, professional organizations, pharmaceutical industries, academicians, patients and patient advocacy groups. These discussions may lead to more efficient, smarter oncology drug development with improved design of future oncology clinical trials.

The first scientific discussion under this project was held on October 8, 2020, in coordination with the American Statistical Association. The topic for this meeting hosted by the American Statistical Association Biopharmaceutical Section was "Type I error considerations in Master Protocols with common control in oncology clinical trials." The panel included academicians, pharmaceutical company representatives, and international regulators.


American Statistical Association with the FDA Forum Discussion held on December 10, 2020 to discuss the Use of Non-concurrent Common Control for Treatment Comparisons in Master Protocols

## PROJECT EQUITY

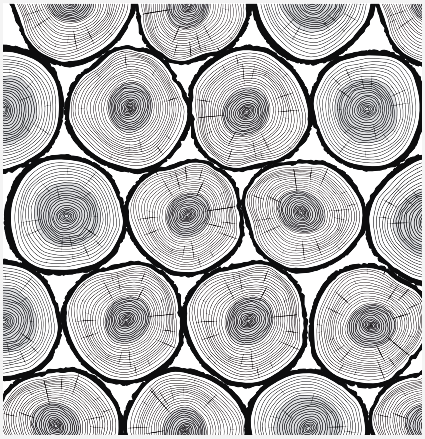
Project Equity is the OCE scientific and policy program to improve evidence generation for underrepresented subgroups in oncology clinical trials. Project Equity's overarching objectives are to 1) provide guidance to industry to facilitate diverse representation in oncology clinical trials, 2) foster collaboration among internal and external stakeholders to promote more equitable and inclusive research and policy practice, and 3) drive research using internal and external data to better characterize outcomes across historically underserved populations in medical research (e.g., racial, ethnic, gender minorities). In 2020, the OCE collaborated with the American Association for Cancer Research (AACR) on the *landmark FDA-AACR Workshop to Examine the Underrepresentation of African Americans in Multiple Myeloma Clinical Trials*, which led to recommendations that were featured in the AACR Cancer Disparities Progress Report. Achievements for Project Equity in 2020 included a symposium entitled "Understanding Barriers to Oncology Clinical Trial Participation for Sexual and Gender Minorities," participation on the American Society of Clinical Oncology (ASCO) and Association of Community Cancer Centers (ACCC) collaboration to foster participation in cancer treatment trials, a prominent feature piece in the ASCO Post titled "*Efforts to Broaden Eligibility Criteria for Clinical Trials Seek to Include More Racial and Ethnic Minority Patients*," among other notable activities.





FDA-AACR Workshop to Examine Under-Representation of African Americans in Multiple Myeloma Clinical Trials, February 13, 2020.
From left: Dr. Paul Kluetz, Dr. 'Lola Fashoyin-Aje, Dr. Nicole Gormley, and Dr. Kenneth Anderson.
Photo by Alan Lessig.

## PROJECT SILVER



Project Silver was launched in 2020 as an OCE strategic program to improve the evidence base for treating older adults with cancer. Through regulatory policy, advocacy and outreach, global engagement, and academic research, the OCE is demonstrating its commitment to drug development for older adults with cancer. This year saw the first Draft Guidance to Industry on Inclusion of Older Adults in Cancer Clinical Trials. The OCE was featured at the 2020 ASCO Educational Session on Global Geriatric Oncology Initiatives. ASCO 2020 also featured an FDA analysis on older men with prostate cancer treated with androgen receptor inhibitors. Under Project Silver, global regulatory agencies will discuss key applications which heavily impact older adults. Looking forward to 2025, with expected growth in the 75 and older population with cancer, the OCE seeks to promote adequate representation of older adults in all stages of cancer drug development.

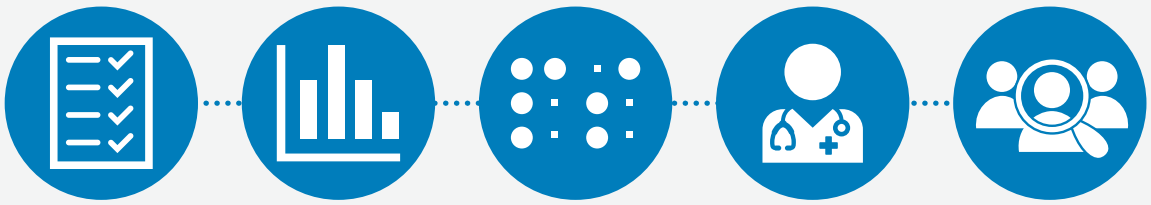
## PROJECT POST COVIDITY

In the COVID-19 era, there is a need to rapidly develop evidence to characterize and treat patients with COVID-19, especially patients in vulnerable populations such as those with cancer. Many clinical trials for COVID-19 therapeutics exclude patients with cancer and similarly, in clinical trials of new cancer therapies the potential impact of the infection on inclusion criteria is unknown; therefore, use of Real World Data (RWD) could inform the conduct and design of clinical trials for oncology drugs. This is a regulatory research opportunity to conduct RWD studies that assist the FDA in characterizing the patient population with cancer and COVID-19. In collaboration with the Friends of Cancer Research and Reagan Udall Evidence Accelerator, Project Post COVIDity is exploring the opportunity to develop RWD parallel analyses among various collaborators to answer specific scientific questions that can provide beneficial knowledge for clinicians and patients.

### OBJECTIVE:

To develop a longitudinal understanding of the impact of COVID on patients with cancer post infection, including the impact of infection on treatment initiation or delays, regimen selection and therapeutic utilization, potential adverse sequalae, and outcomes. The initial focus is on understanding patients with cancer on active treatment and associated safety of immunotherapy.

### POTENTIAL AREAS WHERE PROJECT POST COVIDITY CAN INFORM:



# THE WORK NEVER STOPS: COVID-19

## COVID-19 LISTENING SESSIONS

The Oncology Center of Excellence recognized the opportunity to conduct unique external outreach engagements originating soon after the beginning of the coronavirus pandemic. OCE reached out to oncology stakeholders with COVID-19 Guidance Listening Sessions to better understand COVID-related experiences and the pandemic's impact on cancer patients, advocacy groups and oncology product development. The goal was two-fold: listen directly to cancer patient communities during difficult times and better inform OCE's mission of achieving patient-centered regulatory decision-making through innovation and collaboration. OCE COVID-19 Guidance Listening Sessions occurred with 11 patient, advocacy, and community groups:

**LUNGevity**
April 17, 2020

**Pancreatic Cancer Action Network**
April 20, 2020

**Fight Colorectal Cancer**
April 21, 2020

**Project Community Stakeholders**
April 24, 2020

**National Brain Tumor Society**
May 1, 2020

**Cancer and Aging Research Group**
May 5, 2020

**AACR Scientist Survivor Program**
May 27, 2020

**Melanoma Research Alliance**
June 4, 2020

**National Organization of Rare Diseases**
June 6, 2020

**National Breast Cancer Coalition**
June 9, 2020

**Ovarian Cancer Research Alliance**
September 14, 2020

## #OCEWORKS4U

From the start of the COVID-19 pandemic, OCE leadership wanted to convey to the cancer community that although the FDA's emphasis was on combatting the pandemic, our work in oncology drug development had not stopped or slowed, and patients with cancer were not forgotten. To show our staff's dedication to their work on behalf of patients with cancer, the OCE Communications Program implemented a social media campaign called #OCEWorks4U, featuring photos of staff members working from home and their messages of support for the cancer community. Whether they were medical reviewers, project managers, administrative officers, or associate directors and division directors, the photos of OCE staff members were shared on the @FDAOncology Twitter handle and are visible on the cover of this 2020 OCE Annual Report.


## PROJECT AVATAR

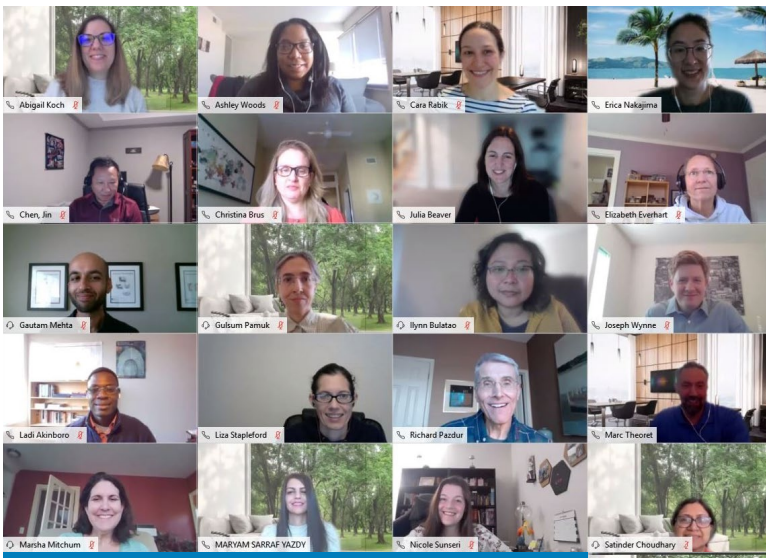
At the end of 2019 and in early 2020, the OCE began a new project to hold some internal meetings entirely virtually due to a shortage of suitable conference rooms and staff working from home or preferring to dial in from their offices. Project Avatar, as this effort was called, was also intended to increase and improve communications with external collaborators and international colleagues. This project was well underway when the COVID-19 pandemic emerged. OCE staff began working from home indefinitely, beginning March 13, 2020. With the experience of Project Avatar, the OCE transitioned relatively smoothly to all-virtual internal and external meetings.

## PROJECT TRACKING

In 2020, OCE launched its first internal tracking database. Due to the extensive activities of the OCE, the database will enable the OCE to keep track of its initiatives and allow for better organization. The database will serve as a central location internal to OCE to track guidances, workshops, presentations, manuscripts and speaking engagements, etc. In addition, the database will provide visibility to ongoing projects and avoid duplicative efforts.

## ONE PILL, ONE LIFE, ONE CAREER

In 2020, the OCE welcomed more than 20 new physicians—oncologists, hematologists, radiation oncologists, and pediatric oncologists—to the FDA from throughout the United States and spanning different stages in their careers. Starting in a new position can be challenging for anyone, and only heightened by the ongoing COVID-19 pandemic and FDA oncology staff working remotely since spring 2020. To help the new OCE physicians assimilate, the OCE established a mentoring program, pairing each person with a mentor within their disease area to help them learn regulatory policy and the nuances around clinical review, and a career development mentor to involve them in OCE projects. The OCE Curriculum also launched with a series of case-based lectures spanning oncology drug development and regulatory policy. A newly established OCE Faculty Seminar Series introduces new FDA oncology physicians to OCE leadership to learn about career pathways and opportunities at the beginning of their OCE careers.



FDA Oncology Welcomes New Reviewers

One pill can transform a life. One life can transform many. One career can transform that pill, that life, that many. Looking ahead to 2025, the OCE looks forward to welcoming those who wish to embark on a new chapter in their transformative careers at FDA oncology.

# RESEARCH

## PROJECT NEXTGEN RESEARCH

FDA's translational research laboratory continues to collaborate with academic oncology centers, NIH, industry, and cooperative groups on projects related to regulatory science. Abstracts on emerging oncology drug targets and acute myeloid leukemia (AML), were presented at prestigious national meetings and genomic work on differentiation syndrome in IDH mutated AML was published. Ongoing projects include using blockchain to transmit genomic data rapidly and securely, examining drug targets for patients with rare tumor types, and exploration of radiomic data to support a better assessment of drug safety and efficacy.

## OCE SCIENTIFIC COLLABORATIVE

The OCE Scientific Collaborative supports FDA oncology staff who participate in regulatory science research, including internal research projects and collaborations with external experts. OCE research focuses on applied (rather than basic) research questions to address specific challenges encountered during the IND and NDA/BLA review process.

OCE staff have contributed to research in many areas of drug development including manufacturing improvements, clinical pharmacology and dose optimization, use of real-world data, effects of expanded eligibility criteria, digital health technology, clinical outcome measures, clinical trial diversity, novel data analysis and visualization methods, and many other key aspects of cancer product development.

Based on a review of internal scientific activities, including publications, invited scientific presentations, public workshops and internal scientific education events, OCE identified nine scientific priority areas and one across-cutting area for research. Additional information about each area can be found on the OCE Scientific Collaborative website.

1. Immuno-oncology
2. Cell /Gene and Personalized Neo-antigen-based Therapies for Cancer
3. Health equity and special populations in oncology clinical trials
4. Oncology trial designs, end points and statistical methodologies
5. Pediatric Oncology
6. Precision Oncology
7. Oncology Patient-Focused Drug Development
8. Oncology Safety
9. Rare Cancers

Developing new approaches to evaluate, integrate, and facilitate the use of oncology real world data was identified as a cross-cutting area that is relevant to several of the scientific interest areas included above. OCE engages in collaborative research with external scientists in these scientific areas using several approaches, including:

- Informal collaborations with academic institutions, for example, to co-publish a scientific commentary
- **Technology transfer agreements** with private companies, academic institutions, and non-profit organizations when data, material (e.g., human biospecimens) and/or financial resources are transferred to the agency to support a research collaboration.
- **FDA Broad Agency Announcement** is a mechanism that FDA uses to solicit research proposals from external institutions. It is open to all responsible sources, including academic, for-profit and non-profit institutions and results in a research contract.
- **Centers for Regulatory Science and Innovation** is a program consisting of cooperative agreements between FDA and six institutions selected through a competitive process (Johns Hopkins, University of Maryland, UCSF-Stanford, and Yale-Mayo Clinic). OCE staff can develop and fund research projects with investigators working in these institutions.

# GUIDANCES

The OCE has led or participated in the development of at least 17 oncology-specific guidance documents in 2020.

| TITLE | TYPE | DATE POSTED |
|---|---|---|
| Cross Labeling Oncology Drugs in Combination Regimens | Draft | November 2020 |
| Premenopausal Women with Breast Cancer: Developing Drugs for Treatment | Draft | October 2020 |
| Renal Cell Carcinoma: Developing Drugs and Biologics for Adjuvant Treatment | Draft | October 2020 |
| Bladder Cancer: Developing Drugs and Biologics for Adjuvant Treatment | Draft | October 2020 |
| Geriatric Information in Human Prescription Drug and Biological Product Labeling Guidance for Industry | Draft | September 2020 |
| Evaluating Cancer Drugs in Patients with Central Nervous System Metastases | Draft | August 2020 |
| Acute Myeloid Leukemia: Developing Drugs and Biological Products for Treatment | Draft | August 2020 |
| Male Breast Cancer: Developing Drugs for Treatment | Final | August 2020 |
| Pathological Complete Response in Neoadjuvant Treatment of High-Risk Early-Stage Breast Cancer: Use as an Endpoint to Support Accelerated Approval | Final | July 2020 |
| Setting Endotoxin Limits During Development of Investigational Oncology Drugs and Biological Products | Draft | July 2020 |
| Cancer Clinical Trial Eligibility Criteria: Patients with HIV, Hepatitis B Virus, or Hepatitis C Virus Infections | Final | July 2020 |
| Cancer Clinical Trial Eligibility Criteria: Patients with Organ Dysfunction or Prior or Concurrent Malignancies | Final | July 2020 |
| Cancer Clinical Trial Eligibility Criteria: Brain Metastases | Final | July 2020 |
| Cancer Clinical Trial Eligibility Criteria: Minimum Age Considerations for Inclusion of Pediatric Patients | Final | July 2020 |
| Developing and Labeling In vitro Companion Diagnostic Devices for a Specific Group or Class of Oncology Therapeutic Products | Final | April 2020 |
| Inclusion of Older Adults in Cancer Clinical Trials | Draft | March 2020 |
| Hematologic Malignancies: Regulatory Considerations for Use of Minimal Residual Disease in Development of Drug and Biological Products for Treatment | Final | January 2020 |

# ENGAGEMENT/OUTREACH

## PROJECT COMMUNITY

Project Community is an OCE national initiative introducing the work of FDA oncologists and hematologists to people in the community, especially under-represented and underserved communities. The primary audience includes patients, advocates, survivors, families and others interested in:

- Increasing minority participation in clinical trials
- Increasing knowledge and minority participation in genetic databases

In 2020 – Project Community organized a Chicago community-based panel discussion in conjunction with Peer Plus Education and Training Associates and the University of Illinois Cancer Center Patient Brigade. The Black History Month cancer awareness panel discussion was entitled "Community Engagement with the Community in Mind; Conversation about the Facts, Figures and Cultural Diversity in Oncology".

Project Community also hosted a COVID-19 Listening Session for community members, patients and advocates. Attending were from University of Illinois at Chicago, Robert H. Lurie Comprehensive Cancer Center of Northwestern University, Peer Plus Chicago, Lombardi Comprehensive Cancer Center at Georgetown University Medical Center, Dana-Farber Cancer Institute/Multiple Myeloma Program, the National Medical Association, University of Chicago Medicine Comprehensive Cancer Center, and the Medical University of South Carolina.


Project Community conducted its first informational session on FDA's Broad Agency Announcement (BAA) cancer research funding mechanism. The video call meeting addressed elements & benefits of the FDA Broad Agency Announcement, OCE's Scientific Interest Areas and how to apply for the funding opportunities. Invited guests included representatives of Historically Black Colleges and Universities (HBCU), Minority Institutions (MI), Small Business and Disadvantaged Business concerns, and others

who are encouraged to submit proposals and to join other entities as team members in submitting BAA proposals.

OCE's Project Community readily participated in the AACR's COVID-19 and Cancer, Health Disparities Forum on July 22, 2020. The virtual panel discussion resulted in the AACR Cancer Prevention Research manuscript, "Disparities in Cancer Prevention in the COVID-19 Era". Excerpt from article:

*"The COVID-19 pandemic, which disproportionately affected minority communities, has curtailed preventive services including cancer screening to preserve personal protective equipment and prevent spread of infection. While there is evidence for a rebound from the pandemic driven reduction in cancer screening nationally, the return may not be even across all populations, with minority population screening that was already behind becoming further behind as a result of the community ravages from COVID-19."*

- John M. Carethers, University of Michigan, Ann Arbor, Michigan Lead Author

## CONVERSATIONS ON CANCER

Conversations on Cancer is a trailblazing and dynamic OCE educational panel discussion series. Riveting cancer-related social issues in the series are inclusive and diverse. 2020 brought exponential growth for OCE's Conversations on Cancer panel discussion series. Groundbreaking at FDA, Conversations on Cancer panel discussions included first of their type program topics focused on Native American, LGBTQ+, Latinx and African American communities and cancer clinical trials in 2020. After successfully building internal audience participant numbers, Conversations on Cancer broadened its reach and is making the robust and engaging discussions open to the public through a free registration process. Informal in tone, the Conversations on Cancer discussion series was originally created by OCE Director Richard Pazdur, MD as an opportunity for internal FDA staff and invited guests to hear from external guests. The tagline "Making Cancer Personal" still holds true and OCE is grateful to all the speaker panelists and audience members for sharing their perspectives and experiences on a wide variety of topics related to cancer.

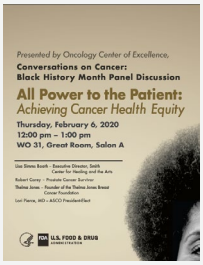
# PANEL DISCUSSION TITLES AND DATES:

## A Dialogue with the HIV and Oncology Communities

January 7, 2020

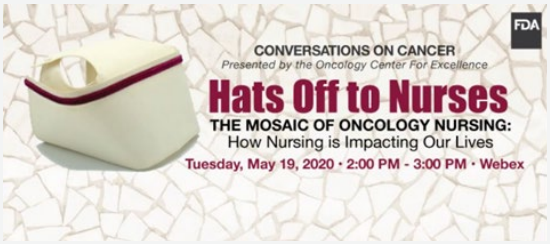
## OCE Black History Month, All Power to the Patient, Achieving Cancer Health Equity

February 6, 2020



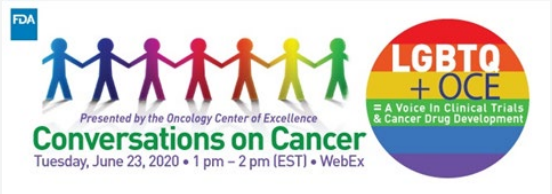

## Hats Off to Nurses, OCE Salutes the Mosaic of Oncology Nursing

May 19, 2020



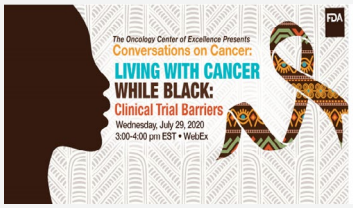

## LGBTQ + OCE = A Voice in Clinical Trials and Cancer Drug Development

June 23, 2020



## Living with Cancer While Black, Clinical Trial Barriers

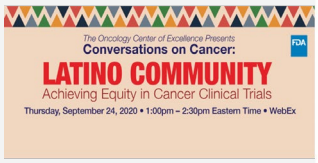
July 29, 2020





## Latino Community: Achieving Equity in Cancer Clinical Trials

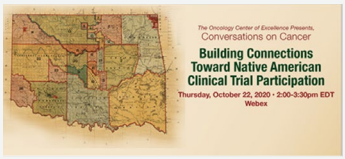
September 24, 2020

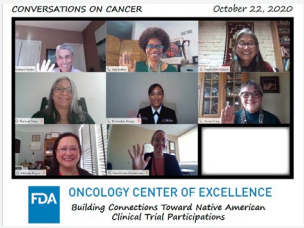




## Building Connections Toward Native American Clinical Trial Participation

October 22, 2020




## Lung Cancer: It Can Happen to Anyone

November 19, 2020


## 2020 PUBLIC WORKSHOPS

- Oncology Center of Excellence...Envisioning Oncology Product Development for 2025
- FDA-AACR Workshop to Examine Under-Representation of African Americans in Multiple Myeloma Clinical Trials
- Tobacco Use in Oncology Care and Research Workshop
- FDA/ASCO Clinical Outcomes Assessment in Cancer Clinical Trials (COA-CCT)
- FDA-ASCO Hematology and Oncology Fellows Day

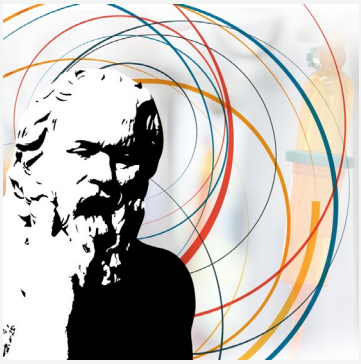
## PROJECT GATEWAY

Through the success of outreach and educational efforts, the OCE has noted greater interest from fellows and oncologists in joining the FDA. Fellows who are still in training frequently have a significant time lag between their OCE interview and their start date, often up to a year. Project Gateway began as an initiative to keep new OCE hires engaged during this time, inviting them to attend educational sessions, public workshops, meetings of the Oncologic Drugs Advisory Committee (ODAC), and many others. In collaborations with Project Socrates and other OCE educational and outreach efforts, the OCE looks forward to welcoming those interested in joining the Project Gateway Class of 2021.

# EDUCATION

## PROJECT SOCRATES

Encompassing OCE's educational initiatives, Project Socrates continues in its second year and continues to strengthen its existing programs and added several new programs in 2020, such as the FDA-AACR Oncology Educational Fellowship, Project Livin' Label, and Icons in Oncology. Looking ahead to 2025 Project Socrates looks forward to bringing the OCE to you through the newly established "On the Road to OCE" program.



## PROJECT LIVIN' LABEL: LABELING UNFOLDED, KNOWLEDGE RELEASED

Project Livin' Label Mascot - Tori

Project Livin' Label is a new OCE educational initiative, in collaboration with the American Association for Cancer Research (AACR), aimed at fostering greater public understanding and awareness of oncology product labels. Indication, dosing, side effects, clinical trial design, and other important prescribing information essential to the safe and effective use of any given product is included in the product label. Using a recent oncology product approval and the associated product label as a roadmap, the OCE will guide discussions between an FDA reviewer, a clinical trial investigator, a patient, a representative from the company, and an international regulator (if the approval was part of Project Orbis) to discuss the backstory behind the product development and the eventual FDA approval. The AACR is offering continuing medical education credit for those who watch these Project Livin' Label episodes and review the associated product labels. Join us as the Project Livin' Label mascot, Tori, takes us through the first two episodes, where we discuss the backstories behind the tucatinib and pemigatinib approvals. All Project Livin' Label episodes can be found here.

## FDA-AACR ONCOLOGY EDUCATIONAL FELLOWSHIP

FDA-AACR Oncology Educational Fellowship, October 15, 2020

The newly established FDA-AACR Oncology Educational Fellowship was announced in July 2020 at the AACR Virtual Annual Meeting II and welcomed 20 hematology/oncology fellows and early-career cancer clinician scientists to its first class. The fellowship consists of six case-based discussion sessions, and span topics in oncology product development studies, to clinical pharmacology and statistical considerations, to early and late phase clinical trial design, and the postmarket setting and beyond. The first session kicked off in October 2020, with additional ones scheduled through spring of 2021. Additional information can be found here.

## FDA-ASCO HEMATOLOGY/ONCOLOGY FELLOWS DAY WORKSHOP


FDA-ASCO Fellows Day Workshop, October 30, 2020

The spring 2020 in-person session of the FDA-ASCO Fellows' Day was postponed due to COVID-19, but welcomed 50 fellows to a half-day virtual session on October 30, 2020. Using recent OCE oncology product approvals as a roadmap, the fellows engaged with OCE physicians in discussions on biomarkers, endpoint considerations, expedited pathways, new drug applications and biologics license applications, investigational new drug applications, and much more. Looking ahead to 2025, the OCE looks forward to continuing its educational and scientific collaborations with ASCO.

## INTERAGENCY ONCOLOGY TASK FORCE FELLOWSHIP

The Interagency Oncology Task Force (IOTF) Fellowship is a collaboration between the FDA, the National Cancer Institute (NCI), National Institutes of Health (NIH), and the Department of Health and Human Services. From 2019-2020, the OCE welcomed one fellow with an interest in malignant hematology and one fellow with an interest in thoracic oncology, each for a year-long fellowship. The fellows participated in longitudinal research projects and actively participated in the regulatory review process, with one fellow ultimately accepting a full-time position with the OCE and starting in November 2020. In October 2020, the OCE welcomed a fellow interested in hematologic malignancies for a year-long fellowship spanning 2020-2021.

## ICONS IN ONCOLOGY

The OCE Icons in *Oncology Distinguished Lecture Series* was established in 2020 to invite esteemed oncologists and scientists to provide a historical perspective of how cancer research, clinical trials, and therapies have evolved over the past few decades. The OCE welcomed Dr. Bruce Chabner on March 2, 2020 and Dr. Norman Wolmark on November 2, 2020.


Icons in Oncology Distinguished Lecture Series, Featuring Dr. Bruce Chabner, March 2, 2020


Icons in Oncology Distinguished Lecture Series, Featuring Dr. Norman Wolmark, November 2, 2020

## OCE PROFESSIONAL SOCIETY LIAISONS

Rapid advances in cancer biology and therapeutics necessitate that FDA maintain an active presence in the scientific community. The OCE scientific liaison program consists of over 20 FDA clinicians and scientists responsible for maintaining a high level of expertise and engagement in multiple disease and research disciplines. Scientific Liaisons engage outside of FDA across focus areas including preventative oncology, health disparities, and integrative oncology as proactive contributors to their field.

In addition to the scientific liaison program, OCE has identified FDA reviewers to serve as Professional Society Liaisons. These FDA scientists support ongoing efforts with leading organizations across the cancer space to further support the OCE mission.

Both of these programs keep OCE abreast of emerging outside opportunities and challenges across fields, enrich FDA staff, and provide a platform to inform important drug development stakeholders of FDA regulations and current thinking.

## OMICS-DRIVEN ONCOLOGY


Many drug development programs in oncology involve genomic analysis, and an increasing number include a companion or complementary diagnostic. The purpose of this educational series was to provide FDA medical officers, statistical reviewers and other scientific staff with a solid understanding of gene and protein-based testing methods commonly encountered by the FDA in sponsor applications. In addition, this educational series addressed interpretation and application of genomic test results in oncology practice and the regulatory framework for the review and approval of genomics-based *in vitro* diagnostics (IVD).

The course included monthly sessions from October 2019 to November 2020. Presenters were be a mix of internal (FDA) and external (non-FDA) experts who delivered the content as in-person presentations or online webinars.

### TOPICS INCLUDED THE FOLLOWING:

| | |
|---|---|
| **Course overview and PCR-based case study** | **Immunohistochemistry and fluorescence in situ hybridization** |
| **DNA and RNA Sequencing** | **Defining the actionable genome: oncogenes and tumor suppressors in the clinic** |
| **Genomic tests in academic and community settings: a tale of two institutions** | **Acquired mutations in solid tumors and how to detect them in the clinic: Breast Cancer** |
| **Acquired mutations in human solid tumors and how to detect them in the clinic: Lung Cancer** | **Acquired mutations in human solid tumors and how to detect them in the clinic: Colorectal cancer** |
| **Acquired mutations in hematological malignancies and how to detect them in the clinic** | **Pharmacogenomics and epigenetics in cancer** |

# PROJECT SOCRATES: THE COLLABORATIVE - FDA-NCI CLINICAL INVESTIGATOR PROGRAM

The FDA and the National Cancer Institute of the National Institutes of Health share a commitment to advancing medical research and developing new or more effective therapeutic agents to treat patients with cancer. The FDA-NCI Clinical Investigator Program is envisioned as one mechanism of interagency collaboration.

The FDA-NCI Clinical Investigator is a clinician-scientist who divides their time between clinical and regulatory duties. They hold a joint appointment in the NCI intramural program serving as an independent, tenure-track, Principal Investigator who develops and conducts cutting-edge clinical trials supported by the NIH Clinical Center and intramural NCI infrastructure. At the FDA, the physician conducts regulatory review work in the Oncology Center of Excellence and the Office of Oncologic Diseases. They develop expertise in U.S. drug and biologic regulation, including novel and transformative therapies. Their clinical trial experience and expertise serve the FDA as "in house" disease specific experts. At the NCI, their regulatory and drug development experience serve the investigator community, IRB, and protocol support office, and academic clinical fellowship program. For additional information, contact FDAOncology@fda.hhs.gov.

FDA U.S. FOOD & DRUG ADMINISTRATION
ONCOLOGY CENTER OF EXCELLENCE

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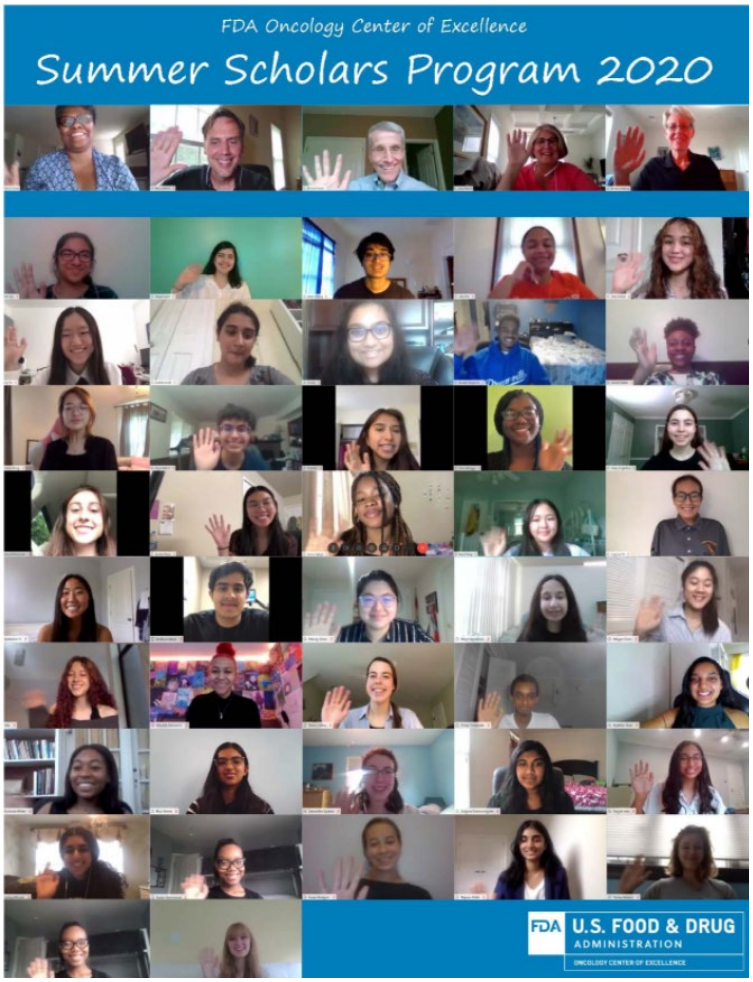
## OCE SUMMER SCHOLARS

The 2020 cohort of OCE Summer Scholars included a record 65 high-school students primarily from DC, Maryland, and Virginia, for the first-ever *virtual* OCE Summer Scholars Program. By shifting to a virtual program, the OCE was able to accept three times as many students as in previous summers.

### The program consisted of:

- Participation in a mock drug development program to follow a product from pre-clinical research through FDA review and marketing.
- Lectures and discussions from experts across the Food and Drug Administration.
- Discussions with other stakeholders and advocacy groups.
- Introduction to principles of translational and clinical research.
- Instruction in communications techniques for future scientists and health professionals.
- Learning about FDA's role in protecting the public health during the COVID-19 pandemic.
- Individual or small-group discussions and mentoring with FDA experts.



# COMMUNICATIONS

The OCE Communications Program supports our leadership and staff in their efforts to share information about the OCE's work to advance the development and regulation of oncology products for patients with cancer. The program coordinates communications with offices across the FDA and with external stakeholders, manages OCE web pages and social media, and assists with articles, talks, guidance documents, journal manuscripts, and research abstracts.

In addition to leveraging other communications where possible, the OCE makes use of its own communications outlets, including:

### OCE WEBSITE ON FDA.GOV:
**http://www.fda.gov/OCE**

### OCE ANNOUNCEMENTS:
**64 APPROVAL ANNOUNCEMENTS**
posted in 2020 on the Oncology/Hematology Approval and Safety Announcements web page. These short articles are also sent via free FDA listserve to more than 90,000 subscribers.

### SOCIAL MEDIA:
The OCE Twitter account, **@FDAONCOLOGY,** has more than **22,500 FOLLOWERS** at the end of 2020.

### PUBLICATIONS:
In 2020, the OCE and affiliated oncology/hematology staff in other FDA centers **PUBLISHED 97 ARTICLES** in scientific journals.

### THE WEEK IN ONCOLOGY:
An internal newsletter for OCE staff and affiliates across the FDA, continued into its third year.

# FDA ONCOLOGY PUBLICATIONS 2020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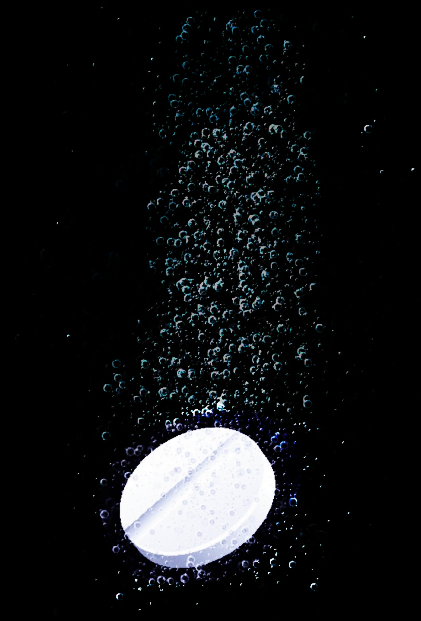

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