

The FDA logo consists of the letters "FDA" in a bold, blue, sans-serif font, enclosed within a white square.

U.S. FOOD & DRUG  
ADMINISTRATION

OFFICE OF NEW DRUGS

**2020**

**ANNUAL REPORT**

March 2021  
[www.fda.gov](http://www.fda.gov)



# Table of Contents

Director’s Message.....	1
OND Organizational Structure .....	2
OND Awards.....	4
OND by the Numbers (CY2020) .....	7
OND COVID-19 Response: Lessons Learned.....	8
Office Snapshots.....	11
Immediate Office (IO) .....	11
Special Programs Staff (SPS) .....	12
Office of New Drug Policy (ONDP) .....	14
Office of Drug Evaluation Sciences (ODES).....	16
Office of Therapeutic Biologics and Biosimilars (OTBB) .....	19
Office of Program Operations (OPO) .....	22
Office of Regulatory Operations (ORO) .....	25
Office of Administrative Operations (OAO).....	27
Pharmacology/Toxicology (Pharm/Tox) .....	28
Office of Infectious Diseases (OID) .....	31
Office of Oncologic Diseases (OOD).....	34
Office of Neuroscience (ON).....	36
Office of Nonprescription Drugs (ONPD).....	38
Office of Specialty Medicine (OSM) .....	41
Office of Cardiology, Hematology, Endocrinology, and Nephrology (OCHEN) .....	43
Office of Immunology and Inflammation (OII) .....	45
Office of Rare Diseases, Pediatrics, Urologic, and Reproductive Medicine (ORPURM).....	48
OND Future Outlook.....	50

Appendix.....	52
Office of New Drug Policy.....	52
Office of Drug Evaluation Sciences .....	52
Office of Therapeutic Biologics and Biosimilars.....	52
Pharmacology/Toxicology.....	53
Office of Infectious Diseases.....	54
Office of Oncologic Diseases.....	55
Office of Neuroscience .....	56
Office of Nonprescription Drugs .....	57
Office of Specialty Medicine .....	58
Office of Cardiology, Hematology, Endocrinology, and Nephrology .....	59
Office of Immunology and Inflammation .....	60
Office of Rare Diseases, Pediatrics, Urologic, and Reproductive Medicine .....	61

## Director's Message

Welcome to the inaugural Annual Report for the Office of New Drugs (OND) in the Center for Drug Evaluation and Research (CDER) of the U.S. Food and Drug Administration (FDA). 2020 was an unprecedented year for OND and FDA. While OND's mission has always focused on ensuring safe and effective drugs and biologics for the American public, our exceptional and dedicated staff shifted their priorities to focus on the COVID-19 pandemic in March 2020 while continuing to tackle existing obligations. OND staff leveraged their expertise and resilience to uphold our commitment to continue delivering impactful drug therapies to the public while prioritizing COVID-19 responsibilities.

In the beginning of 2020, OND's primary focus was completing the implementation of the office's reorganization, which began in October 2019. The OND reorganization was a major undertaking as it restructured many of the clinical offices and divisions, in addition to creating new cross-functional nonclinical offices. As part of this effort, OND staff prioritized recruitment and hiring to help facilitate a smooth transition for these structural changes and ensure effective leadership across all offices and divisions. We worked hard to ensure continuity of all clinical and nonclinical operations throughout the reorganization and transition of our offices and staff.

Our priorities quickly shifted with the outbreak of the COVID-19 pandemic as it forced the staff to react swiftly and effectively to fulfill evolving public health needs. OND's response to the pandemic saw increased collaboration across offices and divisions to support and accelerate development programs for new therapeutics to prevent or treat COVID-19. Despite the added workload, there were no significant disruptions to OND's work as staff managed to meet and/or exceed many of the Prescription Drug User Fee Act (PDUFA) review goals, approve 53 novel drugs, publish 31 new guidances, and maintain regulatory and programmatic operational responsibilities under tight deadlines and difficult circumstances.

Our 2020 Annual Report highlights the accomplishments of OND staff and provides insights into the operations of OND's offices and divisions. OND's success this past year is entirely the result of our resilient and devoted staff, whose work during these unprecedented times of the COVID-19 pandemic has made a major difference in OND's response. I am proud to be part of such an extraordinary team of individuals who continue to advance the vision of OND.

I look forward to continuing our mission in 2021.

**Peter Stein, M.D.**

Director, Office of New Drugs



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OND is comprised of  
over 1,300 employees!

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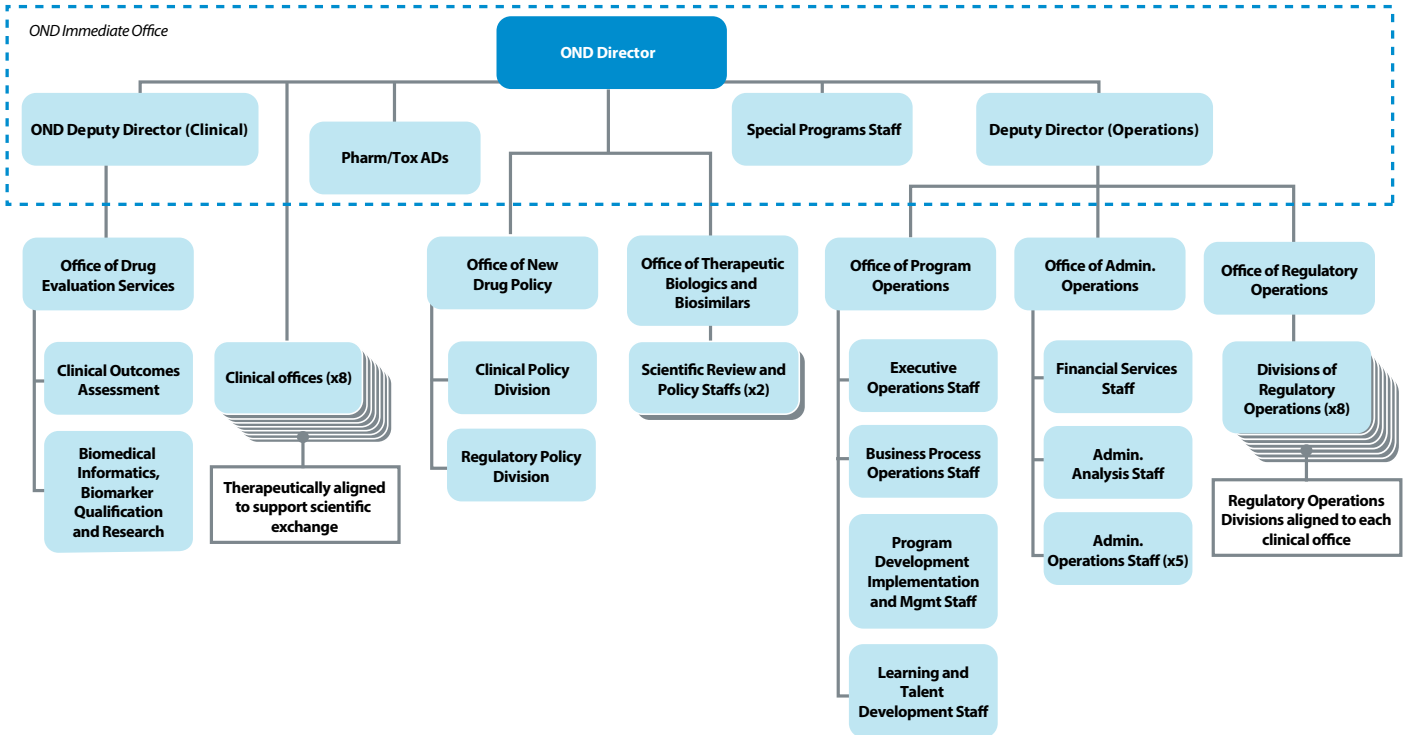
## OND Organizational Structure

[OND's role](#) is to regulate the development and approval of new drugs and new uses of already approved drugs, and to monitor the safety of drugs. This work includes reviewing drug applications, providing guidance to pharmaceutical companies on the development of drugs, working with other external stakeholder groups on drug development issues, and determining whether the benefits of drug therapies outweigh their known risks throughout the lifecycle of the drug – during development, during review for approval, and post-approval.

The New Drug Regulatory Program (NDRP) includes all the CDER offices that are involved in new drug review and regulation, including OND, the Office of Translational Sciences (OTS), the Office of Surveillance and Epidemiology (OSE), and the Office of Product Quality. In 2017, CDER began an initiative to modernize the NDRP. One element of the modernization was an [organizational restructuring](#) of OND to, among other things, more appropriately align offices and divisions by interrelated disease and therapeutic areas, with additional changes to other NDRP offices.

The intent of the realignment was to create offices focused on broad therapeutic areas (such as neuroscience or inflammation/immunology), and to create divisions that are more disease area-focused. These changes to OND's structure resulted in eight clinical offices that oversee 27 review divisions, plus six nonclinical review divisions. In addition, the reorganization created a series of six "infrastructure" offices, providing support across all the clinical offices and divisions. These infrastructure offices are intended to provide services, such as advice on policy issues or on drug evaluation tools or support for regulatory operations. By working across clinical offices, these new infrastructure offices can develop greater expertise, enhance consistency of approach, and strengthen transfer of new learnings from one clinical office to another. This new office and division structure better distributes high volumes of work and affords leadership more time to invest in developing employees while reaffirming the commitment to strengthening program operations and the science behind drug evaluation. The reorganization also created new divisions of Pharmacology/Toxicology (Pharm/Tox) in the clinical offices, enhancing the role of this key discipline, and supporting greater cross-divisional work and collaboration. The reorganization furthers talent retention by enabling enhanced career paths and introduces new roles that modernize and strengthen OND's work.

# High-Level OND Organizational Chart





## OND Awards

### 2020 FDA Honor Awards

FDA honor awards are non-monetary awards that recognize cross-cutting accomplishments and significant contributions of individuals or groups that have supported the Agency's goal of meeting its mission of protecting and promoting public health both on a national and international level.

Below are several types of FDA honor awards that OND staff received for their noteworthy contributions to protect the public health of the American people, as well as recognition for the extensive years of service dedicated to serving FDA and its mission.

#### FDA Group Recognition Award

##### Post Approval Pregnancy Safety Studies Guidance Work Group

*For outstanding work in developing a draft guidance that outlines the appropriate methods of safety data collection in pregnant women in post-approval safety studies.*

- Denise Johnson-Lyles, Ph.D.
- Robert Levin, M.D.
- Leyla Sahin, Ph.D.
- Jacqueline Yancy, Ph.D.
- Lynne P. Yao, M.D.



### Sunscreen Proposed Rule Team

*For outstanding collaborative efforts on the 2019 FDA issued Proposed Rule updating requirements on sunscreen products.*

- Steven A. Adah
- Francis E. Becker
- Paul Brown, Ph.D.
- Sergio Coelho
- Teegan A. Dellibovi-Ragheb
- Gordana Diglisic
- Elizabeth Donohoe, M.D.
- Jane Filie
- Charles J. Ganley, M.D.
- Wafa Harrouk, Ph.D.
- Chibueze A. Ihunnah
- David L. Kettl
- Mona Khurana, M.D.
- Kendall Marcus, M.D.
- Melinda L. McCord
- Theresa Michele, M.D.
- Steven F. Osborne
- Miya O. Paterniti
- Jane Sohn, Ph.D.
- Arlene H. Solbeck
- Jian Wang
- Jennifer J. White
- Andrew Zacher, J.D., M.S.

### FDA's Commissioner's Special Citation

#### **Trikafta Review Team**

*For upholding the FDA's public health mission by making available a safe and effective treatment for patients with Cystic Fibrosis.*

- Sandy Barnes
- Stacy J. Chin
- Andrew C. Goodwin
- Courtney S. McGuire
- CDR Angela Ramsey
- Sally Seymour, M.D.
- Dong Zhao

### FDA Public Health Service Commissioned Corps Awards

#### **Outstanding Service Medal**

*For outstanding leadership and coordination of the FDA's, DAVP's regulatory response to the 2018 Ebola Virus Disease Outbreak.*

- LCDR Andrew Gentles

*For performance of a heroic and brave act by administering Cardiopulmonary Resuscitation (CPR) to a man suffering from cardiac arrest on September 1, 2017.*

- LCDR Ji Hyun LaRose

#### **FDA Alumni Association FDA Innovator Award**

*For outstanding efforts to modernize the new drugs regulatory program.*

- Kevin Bugin, Ph.D.

### **Award of Merit**

*For exceptional leadership in promoting FDA's fundamental core values, using FDA's "flexible" model to speed review and access to new therapies for pediatric cancer.*

- Martha Donoghue, M.D.

### **50 Years Length of Service**

- Doris E. Garrison
- Robert Temple, M.D.

### **40 Years Length of Service**

- William B. Tauber, M.D.

## **2020 OND Awards**

A new program (2020 was the inaugural year) has created a series of OND awards that are to be given to recognize staff achievements and contributions to OND's mission, as well as exceptional performance and service. Eventually, four awards are planned, with two so far awarded.

### **Eric G. Colman Award for Excellence in Scientific Communication**

*In memory of Eric G. Colman, M.D., this award was established to recognize a staff member within OND who effectively communicates scientific findings and regulatory decisions made by FDA to the broader public.*

- Armaghan Emami, Ph.D.

### **Excellence in Meeting Our Public Health Mission**

*This award recognizes OND individuals or groups who went above and beyond to demonstrate innovation and leadership over the past year to address an important public health need affecting our country, substantially advancing OND's mission. The inaugural award recognized the many offices and divisions across OND that have played key and essential roles in our response to the COVID-19 pandemic.*

- Division of Antivirals and the Office of Infectious Diseases
- Division of Rheumatology and Transplant Medicine
- Office of Oncology Drugs
- COVID Triage/ Jurisdiction/ COVID Scientific Technical Triage Team
- Division of Pulmonology, Allergy, and Critical Care
- Office of Nonprescription Drugs
- Deployed Public Health Service Officers
- Individuals Detailed to Other Divisions to Support COVID-19 Workload
- Division of Cardiology and Nephrology
- Office of New Drug Policy
- Office of Program Operations
- Chief Project Management Staff

## OND by the Numbers (CY2020)

OND demonstrated a successful year for novel drug approvals, industry meetings, and guidances during calendar year 2020. Please see below for more information.

[INDs Received](#)

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[Breakthrough Therapy Requests](#)

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[INDs with Activity](#)

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[Fast Track Requests](#)

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[Novel Drug Approvals \(NME NDAs/BLAs\)](#)

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[Expanded Access INDs Received and Safe to Proceed](#)

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[NDA and BLA Approvals](#)

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**3,571**

Industry Meetings (Scheduled)\*

\*Count is for OND meetings only and includes PDUFA and BsUFA meetings.

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[Efficacy Supplement Approvals](#)

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**31**

Published Guidances

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## OND COVID-19 Response: Lessons Learned

The outbreak of COVID-19 presented an unprecedented challenge to OND in 2020. The extensive efforts by the pharmaceutical industry, academic investigators, and the National Institutes of Health (NIH) to find therapeutics for COVID-19 led to a sudden increase in workload for staff across OND. The additional workload included reviewing submissions for potential COVID-19 related therapies, answering inquiries from external stakeholders, and addressing various policy issues. OND staff worked relentlessly to review submissions and respond to applicants and other stakeholders, tirelessly providing advice and collaborating with sponsors, academics, and companies to progress promising therapies. COVID-19 threatened to disrupt numerous ongoing clinical trials for other indications, so OND divisions worked quickly with sponsors to manage and modify those trials to ensure they could continue in a new, largely virtual environment. Despite these increased COVID-19 responsibilities, OND and the NDRP continued to meet its PDUFA obligations, and in some cases, met PDUFA review deadlines ahead of schedule.

Part of OND's response involved assessing new and existing therapeutics that showed promising results in COVID-19 patients. OND used Emergency Use Authorizations (EUAs) to bring promising therapies to the American public more rapidly. Under Section 564 of the Federal Food, Drug, and Cosmetic Act, FDA may allow unapproved medical products or unapproved uses of approved medical products to be used in an emergency to diagnose, treat, or prevent serious or life-threatening diseases or conditions caused by chemical, biological, radiological, or nuclear threat agents where there are no adequate, approved, and available therapies. OND divisions contributed to the authorization of [seven EUAs for COVID-19](#) between March and December 2020: chloroquine phosphate and hydroxychloroquine sulfate (subsequently revoked on June 15th); Veklury (remdesivir); MultiBic; Propoven 2%; Regiocit; Bamlanivimab; baricitinib (Olumiant); casirivimab and imdevimab. The EUA for remdesivir paved the way for its subsequent approval in October 2020 as the first FDA-approved treatment for COVID-19.

OND Policy (ONDP) led the development of the Coronavirus Treatment Acceleration Program (CTAP), FDA's special emergency program to explore and determine possible coronavirus therapies. The program expedited the development of potential COVID-19 therapeutics and moved new treatments to patients as quickly as possible. CTAP created a [dashboard](#) to visually depict a snapshot of the development of potential

COVID-19 therapeutics. As of December 31, 2020, OND reviewed over 320 trials for potential COVID-19 therapeutics and over 570 drug development programs in the planning stages.

OND significantly contributed to 10 COVID-19-related guidances from March through December 2020 (including three COVID-19 guidances authored by OND). These included [guidance on the pre-IND meeting request content for COVID-19 drugs or biologics](#), as well as [guidance on the development of drugs and biologics to treat and prevent COVID-19](#), including population, trial design, efficacy endpoints, safety considerations, and statistical considerations. OND also led the development of another [guidance on formal user fee applications and meetings](#) in collaboration with CDER and the Center for Biologics Evaluation and Research (CBER). The [COVID-19 guidance on conduct of clinical trials](#) also included significant contributions by OND staff.

Another area of significant work on guidances for the COVID-19 pandemic included the development and [publication of several guidances on hand sanitizer safety](#). Early in the pandemic, the Office of Nonprescription Drugs (ONPD) led extensive efforts to ensure the availability of safe alcohol-based hand sanitizers, working with other offices across CDER in this huge effort. These guidances were focused on informing industry and the public of safety measures regarding the manufacturing and use of hand sanitizer products.

When OND leadership realized a few divisions were disproportionately impacted by COVID-19 related inquiries, OND launched a COVID-19 product development mailbox as the initial entry point for inquiries and submissions. This required constant project management support to monitor the inbox and rapidly triage COVID-19 related submissions. With the outbreak of the pandemic, OND saw a significantly higher volume of submissions from individuals and entities with no prior regulatory experience. In response, OND created the COVID Scientific Technical Triage Team (CSTTT) to work closely with these sponsors. CSTTT drew members from across OND and worked to prepare submissions and ensure applications had sufficient and critical information for divisions to complete a review. A jurisdiction team was created to assign submissions to the appropriate division. OND's Office of Regulatory Operations (ORO) also helped rapidly adapt OND's record and archival system to enable virtual submissions instead of paper submissions during a time of increased telework by both FDA and industry.

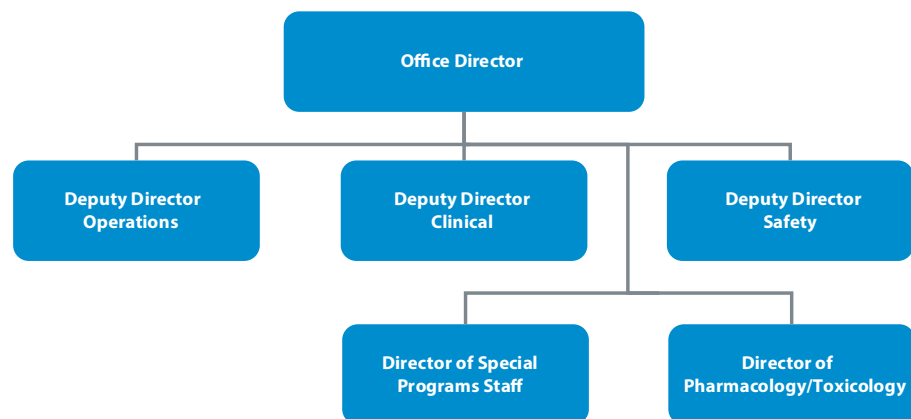
In response to the increased workload for COVID-19 therapies, volunteers from throughout OND stepped in to provide support. Medical reviewers, nonclinical reviewers, clinical analysts, and project managers volunteered to support CSTTT, as well as the OND divisions most impacted by the onslaught of COVID-19 submissions, including the Division of Antivirals (DAV), the Division of Pulmonology, Allergy and Critical Care (DPACC), and the Division of Rheumatology and Transplant Medicine (DRTM). OND staff continued to effectively leverage division consults to support COVID-19 application reviews and offer therapeutic expertise, as needed. A key factor to OND's successful response to COVID-19 was the collaboration to help support colleagues most impacted by COVID-19-related work.

OND's response to the COVID-19 pandemic demonstrated the ability of OND staff to continue operations despite the transition to fully remote work. Staff proved their adaptability in working virtually, finding ways to enhance productivity and engagement in virtual meetings, and identifying solutions to rapidly adjust processes and information-sharing mechanisms to the new work environment. In the transition to fully remote work, OND continued to hire and onboard new employees across offices and divisions, which was particularly critical due to OND's reorganization. Staff quickly developed virtual processes for interviewing and onboarding that provided a smooth process for interviewees and new hires to OND.

Across OND, staff demonstrated their dedication to OND's mission and improving the health of the American public as they shouldered their normal work, as well as the surge of COVID-19 related work throughout 2020. Ultimately, it was the hard work of OND's staff who made the difference in FDA's strong COVID-19 response and demonstrated the true resilience of the organization.

## Office Snapshots

### Immediate Office (IO)



The IO provides high-level supervisory support across the organization to help OND offices and divisions carry out their mission to provide safe and effective new drugs to the American public. To accomplish this, the OND IO supports a weekly senior OND and CDER management meeting to discuss broad policy and IND program issues, responds to formal dispute resolution requests, works to assure appropriate staffing and related policies, and meets regularly with clinical office and divisional leadership to provide input regarding ongoing challenging regulatory issues. In addition, the senior Pharm/Tox staff is situated in the IO. These highly experienced individuals provide supervisory input across the OND Pharm/Tox divisions and focus on policy and program development related to Pharm/Tox. A new staff within the IO providing support for safety data analysis is being built. This staff, Clinical Data Scientists, are providing standardized safety reports to teams reviewing New Drug Applications (NDAs) and Biologics License Applications (BLAs) and supporting needed additional safety analyses. This team was created in 2018, and has markedly expanded its capacity, providing support for 30 NDAs/BLAs in 2020.

## Special Programs Staff (SPS)

Office Director

Deputy Office Director

SPS designs, coordinates, develops, and collaborates on the implementation of quality improvement of high-priority OND initiatives that are essential to achieving the mission of OND and the NDRP. NDRP is the modernization effort that OND is currently undergoing to better serve patients and better support staff; this effort builds on OND's past successes and strengths by implementing problem-focused, interdisciplinary, team-based approaches to meet the challenges of evolving science, new drug platforms, and new drug targets while incorporating the patient voice in development. SPS supported the implementation and evaluation of several NDRP Modernization workstreams in 2020: Advisory Committees, Knowledge Management, Integrated Assessment of Marketing Applications, and Postmarket Safety.

The Advisory Committee workstream was established and tasked with developing a roadmap to ensure OND Advisory Committee meetings provide well-informed, clear, and consistent expert advice to internal and external stakeholders on challenging scientific, regulatory,





technical, and policy matters. In 2020, SPS supported OND's transition to virtual Advisory Committee meetings with the COVID-19 pandemic. SPS identified feasible options for conducting virtual meetings, worked with OND divisions to determine the best option for a given meeting, and gathered feedback to ensure the efficiency and success of future virtual Advisory Committee meetings.

With knowledge being OND's primary asset, the Knowledge Management workstream functions to provide strategy, tactical guidance and oversight for knowledge management initiatives and data governance across the NDRP. The workstream commenced its work with gathering, ideating, and prioritizing more than 200 NDRP knowledge management requirements for further development and implementation. Once implemented, it will significantly improve OND's ability to retrieve and utilize information for regulatory decision-making and accountability. The workstream closely partners with CDER-wide information, workflow, and data management leadership and development teams to support a unified strategy for addressing knowledge management needs across CDER.

Another SPS priority was the development of Integrated Assessment of Marketing Applications, which enhances interdisciplinary collaboration in OND's review process, improves the clarity of the final review documents, and more effectively communicates the basis for FDA's decision on approval or denial of drug applications to appropriate audiences.

SPS also played a key role in Postmarket Safety, which introduced and implemented several Drug Safety Teams (DSTs) in 2020. The goal of the Postmarket Safety workstream, which is comprised of SPS, CDER's Drug Risk Management Board, and OSE, is to develop a standard process for collaborative, interdisciplinary scientific safety expertise to facilitate efficient sharing of information about safety issues across the Center. The DSTs are comprised of individuals with different areas of expertise to survey what other offices are doing, allowing the teams to collaborate to better understand, identify, and address safety issues. The DSTs are a new structure, with the initial DSTs set up to learn how to best organize and run these DSTs to be efficient and enhance cross-office collaboration. The DSTs are still a "work in progress."

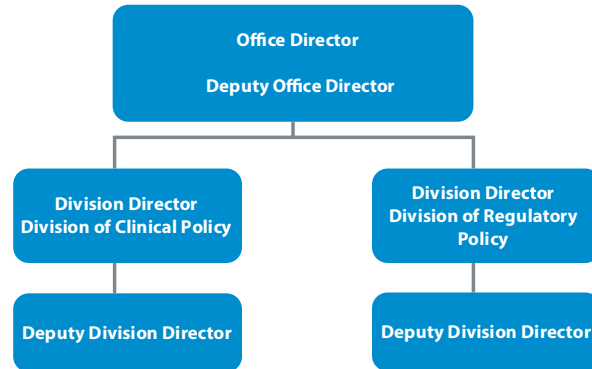
Across all these efforts in 2020, SPS collected feedback from internal staff and external stakeholders regarding the strengths and areas of improvement for these new processes and tools. SPS' review helps ensure the successful development, implementation, and durability of the NDRP Modernization effort.

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In 2020, SPS assisted in the completion of 17 Integrated Reviews and has 19 ongoing marketing application reviews that utilize the Integrated Review template and new process developed under the NDRP modernization.

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## Office of New Drug Policy (ONDP)



ONDP was created in 2019 as part of the NDRP Modernization reorganization to provide OND with a dedicated policy function. The office advises review teams concerning specific legal, regulatory, clinical, and scientific issues that arise across the NDRP, including in drug development, drug labeling, submission review, and expanded access programs. ONDP also expedites the development and clearance of OND guidances, including disease- and indication-specific guidances, manages the 505(b)(2) program, which provides regulatory oversight and consistency to 505(b)(2) NDAs, coordinates policy development across OND, standardizes policy across therapeutic areas, and modernizes policy to meet emerging scientific challenges. ONDP is comprised of two divisions: the Division of Regulatory Policy and the Division of Clinical Policy.

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OND Policy managed and cleared 29 OND-authored guidances that published in CY 2020!

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ONDP's informal motto is, "we are here to help." To that end, the office works to solve policy problems, answers policy questions, and navigates FDA's legal and policy network, enhancing review efficiency and enabling OND's scientists to focus on the science. ONDP's multidisciplinary staff includes physicians, scientists, pharmacists, regulatory affairs experts, and lawyers.

When COVID-19 struck, ONDP hit the ground running. The ONDP team led the development of CTAP and staff led or participated in several COVID-19 policy development efforts, including guidances on clinical considerations for COVID-19 drug development programs. ONDP staff provided integral policy support services to OND's clinical review divisions in connection with OND's review and consideration of EUA requests for COVID-19 therapeutics. Some of ONDP's staff became frontline reviewers for OND divisions heavily impacted by COVID-19,

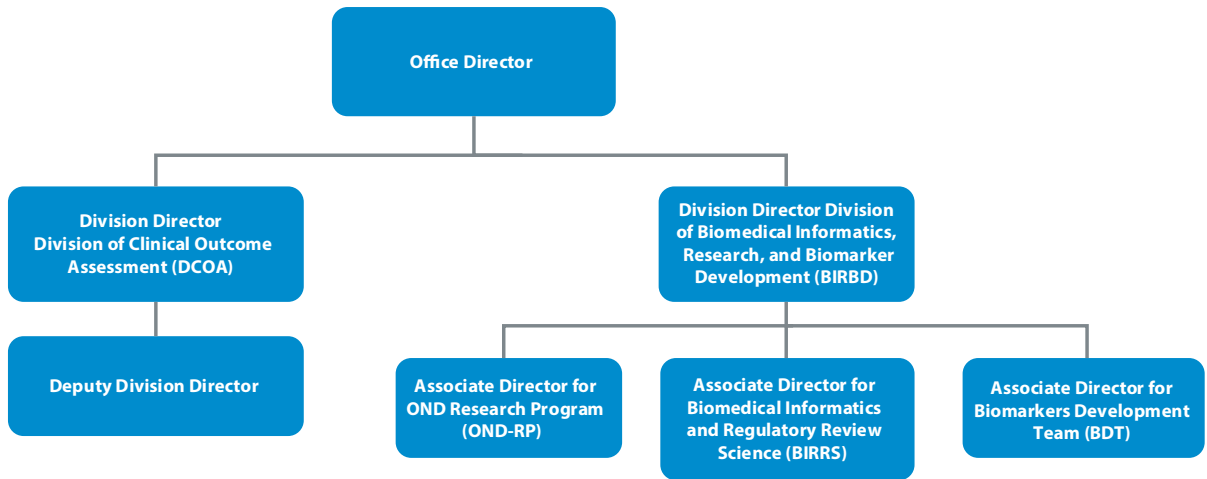


while other ONDP staff stepped in to support the triaging of COVID-19 related submissions and EUAs. Several ONDP staff participated in deployments as part of the Public Health Service's response to the pandemic.

While balancing COVID-19 related work, the ONDP team pushed forward on existing strategic policy priorities cutting across disease areas. These priorities included: developing a guidance pertaining to clinical evidence of effectiveness and a guidance pertaining to confirmatory evidence; improving existing drug benefit-risk policy; developing an interim policy framework for individualized medicine; and developing and implementing improvements to the 505(b)(2) NDA program. ONDP's (b)(2) team cleared a record number of 505(b)(2) application actions, as OND's 505(b)(2) program continues to grow annually.

ONDP is steadfast in its commitment to OND's mission. To that end, ONDP focused on developing strong collaborative relationships throughout OND, CDER, and FDA so the office can continue to help OND offices and divisions meet PDUFA review goals and other obligations to better serve the American people.

## Office of Drug Evaluation Sciences (ODES)



ODES was created by the OND reorganization to promote innovation in drug evaluation science to facilitate the availability of new drugs that are safe and effective for their intended use. ODES includes the Division of Clinical Outcome Assessment (DCOA) and the Division of Biomedical Informatics, Research, and Biomarker Development (BIRBD). BIRBD is comprised of three teams: Biomedical Informatics and Regulatory Review Science (BIRRS), the OND Research Program (OND-RP), and the Biomarkers Development Team (BDT). ODES and its divisions collaborate extensively with internal and external stakeholders to provide expertise, support, and tools to OND clinical offices to facilitate drug development and review.

DCOA manages the Clinical Outcome Assessments Qualification Program and works with OND review divisions and CDER's Office of Biostatistics (OB) to evaluate the clinical benefits and/or harms of therapeutics by ensuring that what is important to patients, caregivers, and clinicians is captured using well-defined and reliable COAs in clinical trials. In 2020, DCOA participated in 547 consults from OND review divisions and other FDA Centers. DCOA led the development of a [guidance](#) covering how to measure/analyze COVID-19 symptoms in trials of therapies to treat/prevent the disease, including patient-reported outcomes. DCOA also participated in the development of four guidance documents related to patient-focused drug development (PFDD) that aim to enhance methodologic rigor around obtaining representative patient and caregiver input and developing/selecting patient-centric COAs and associated endpoints.

The BIRRS team finalized and launched two safety analytics initiatives to help clinical review teams detect potential safety signals during reviews: FDA Medical Queries (FMQs) and Standard Safety Tables and Figures (ST&F). To launch the new standards, the BIRRS team hosted multiple virtual trainings to provide background and instruction on how OND reviewers can access and use both FMQs and ST&F when conducting NDA reviews. The sessions were attended by over 600 participants and received favorable feedback from attendees, so OND extended the training's availability by offering online recordings to allow more FDA staff to learn about and implement the two safety analytics initiatives.



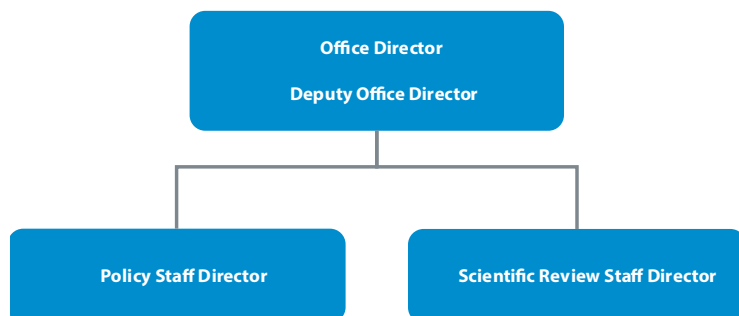
## BIRRS Team Meeting

OND-RP was established in 2018 to 1) provide oversight for office-level spending on regulatory science research (RSR) and 2) educate and support OND staff conducting RSR projects. OND-RP manages the OND Research Committee (ORC), which peer-reviews and prioritizes all OND RSR projects submitted for funding each fiscal year (FY). In the ORC proposal review process for FY21, 24 out of the 40 proposals submitted to OND were ultimately funded. OND-RP organized two Open Houses to raise awareness of professional development resources and opportunities for OND's 41 Oak Ridge Institute for Science and Education (ORISE) fellows. In November 2020, OND-RP hosted a public webinar to advertise OND fellowship opportunities available through the ORISE Program and to raise awareness of two OND-led Congressionally mandated programs that have funding available to support collaborative research with external investigators. This [webinar](#) was attended by 291 attendees from 51 countries.

The BDT manages CDER's Biomarker Qualification Program (BQP), which has over 50 active projects spanning many of OND's clinical disease areas. In 2020, the team focused efforts to develop improved biomarkers for drug-induced organ toxicity and diagnostic enrichment in Non-Alcoholic Steatohepatitis (NASH) clinical trials. BQP collaborates with OND's clinical divisions in the updated public release of the Surrogate Endpoint Table every six months and is writing a guidance to define the types of evidence needed to support use of surrogate endpoints. In December 2020, the BDT launched the [Innovative Science and Technology Approaches for New Drugs \(ISTAND\)](#), a new pilot to provide scientific and logistical support to drug development tool (DDT) developers and FDA's clinical divisions in assessing novel technologies and scientific approaches that have the potential to advance drug development and speed new therapeutics to patients.

In 2020, the Drug Trials Snapshot (DTS) group transitioned to ODES from the Professional Affairs Stakeholder Engagement group within CDER's Office of the Center Director. Snapshots are a summary of information and data from clinical trials that include sex, age, race, and ethnicity, as well as efficacy and safety among these subgroups. Transitioning DTS to OND enables early involvement of DTS staff in the review of NDAs and original BLAs as an integrated member of the review team. Besides providing a high-level summary of demographic data from clinical trials, the snapshots also provide information to the medical and scientific community to consider where data gaps exist to ensure that future clinical trials will adequately represent the diversity of U.S. patients. Since DTS' creation in January 2015, FDA has published over 282 DTSSs.

## Office of Therapeutic Biologics and Biosimilars (OTBB)



OTBB is comprised of 25 staff members who coordinate and support all FDA activities related to biosimilar and interchangeable product development and approval. OTBB consists of: (1) an IO that leads OTBB and biosimilar review program strategy, internal and external communications, and coordinates Biosimilar User Fee Act program activities; (2) the Scientific Review Staff, who provide oversight and ensure consistency in the multi-disciplinary review of biosimilar/interchangeable products, conduct regulatory research, and facilitate the resolution of novel scientific issues associated with biosimilar/interchangeable products; and (3) the Policy Staff, who generate policy and guidance regarding biosimilar/interchangeable products and help to identify, evaluate, and resolve regulatory issues.

In 2020, OTBB led OND's progress towards all four goals in FDA's Biosimilars Action Plan (BAP) and accomplished several key deliverables outlined in the BAP. This included the release of several draft and final guidances to support the efficiency, quality, and predictability of biosimilar product review and development. OTBB also held public meetings to hear from stakeholders about biosimilar and interchangeable products, released Spanish language educational materials for patients, enhanced the Purple Book, and developed new review templates to improve efficiency and consistency in the biosimilar review process.

Supporting a competitive marketplace for biologics, including biosimilar and interchangeable products, is essential for expanding patient access to medicines and reducing health care costs. To this end, FDA is collaborating with the Federal Trade Commission (FTC) to outline shared goals and objectives to guide how the agencies will work together to promote competitive markets for biological products and take appropriate action to address false and misleading statements



and promotional communications by biologics manufacturers. OTBB, along with the Office of Prescription Drug Promotion and the Office of Regulatory Policy, held a joint public workshop with the FTC in March 2020 to advance the BAP goal of supporting market competition by reducing gaming of FDA requirements or other attempts to unfairly delay competition. Ahead of the workshop, FDA released a [guidance](#) to support this goal and ensure that prescription drug information is truthful, balanced, and accurately communicated. OTBB contributed to another [guidance](#) to provide information about FDA's thinking on comparative clinical immunogenicity studies for proposed biosimilar or interchangeable insulins.

Over the past three years, OTBB has completely overhauled the [Purple Book: Database of FDA-Licensed Biological Products](#), transitioning it to a searchable, online, public-facing database that improves access to timely information about biological products. FDA's enhanced Purple Book Database includes more robust data about each product entry, like product presentation(s), strength(s), dosage form(s), and available exclusivity information. It fulfills a key action in the BAP to develop an enhanced Purple Book that includes information about newly approved or withdrawn BLAs, in addition to available exclusivity information and helps support OTBB's mission to increase innovation and competition among biologics and to encourage the development of biosimilars.



Internally, OTBB was hard at work finalizing biosimilar-specific multidisciplinary review templates, which seek to reduce reviewer burden by improving the efficiency and consistency of the review process for evaluating marketing applications for biosimilars. Review teams have piloted the templates for five recent approvals. This has been a successful effort thanks to the collaborative efforts of several OND offices that piloted and provided valuable feedback which OTBB is applying to refine the templates.

The transition to an all virtual environment was relatively seamless for OTBB, and the office continued to meet with OND review teams effectively and without delaying timelines. In particular, the virtual environment enabled OTBB to engage and increase outreach to stakeholders, in part because OTBB staff were more easily able to attend and present at external stakeholder meetings since there was no need for travel time. After FDA moved to a largely virtual environment in March 2020, OTBB staff presented at several meetings including the American Autoimmune Related Diseases Association, America's Health Insurance Plans, and the ERISA Industry Committee. OTBB made inroads with employer and insurance groups, which is exciting because insurance/payer coverage of biosimilars can greatly impact biosimilar uptake and prescribing. OTBB's Policy and Scientific staff have also remotely presented at several industry, regulatory, and academic conferences about biosimilar and interchangeable products, furthering OTBB's education efforts.

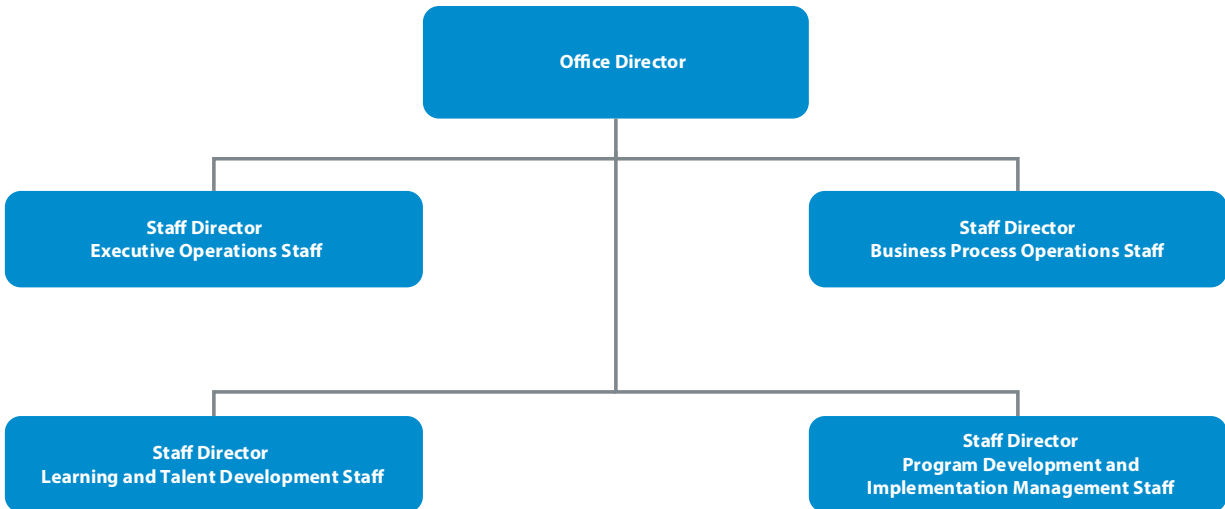
Despite COVID-19 and the transition to a fully remote work environment, OTBB made significant progress towards reaching FDA's BAP goals in 2020.

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OTBB staff released materials in Spanish to increase awareness about biosimilars among Spanish-speaking patients. The new materials include Spanish versions of the 'What is a Biosimilar?' infographic for health care providers and the Biosimilars Basics website and infographic for patients and the biosimilars consumer update.

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## Office of Program Operations (OPO)



OPO collaborates with staff and leadership across CDER and OND to support the execution of a wide range of new drug programs and business process activities. OPO also provides learning and training opportunities for OND staff and provides support for Executive-level initiatives. OPO is a newly formed office created during the OND reorganization. The office maintains four staffs: the Business Process Operations Staff (BPOS), the Executive Operations (ExecOps) Staff, the Program Development, Implementation and Management Staff (PDIMS), and the Learning and Talent Development (LTD) Staff.

BPOS oversees OND's business process operations to support CDER's mission and goals and collaborates with process owners to enhance consistency and efficiency. In 2020, BPOS was integral in the development and implementation of four CDER New Drug Review workflows.

BPOS played a key role in FDA's COVID-19 response. The staff contributed to the development of and updates to CDER CTAP metrics, CDER NextGen Portal (e.g. Manufacturing Capacity, EUA), COVID-19 Data Lake that holds all COVID-19 related data, Accelerating COVID-19 Therapeutic Interventions and Vaccines (ACTIV) Clinical Trials Dashboard, and OND's internal COVID-19 Dashboard. BPOS also made updates to CDER and OND processes in response to the pandemic. BPOS served as the OND communication conduit for COVID-19-related metrics and information internally to the CDER COVID-19 Task Force, CDER Senior Leadership, CDER Director, and OND Director, and for external sources.

The ExecOps Staff provides support to a variety of critical initiatives for OND Executive Leadership and serves as the liaison between CDER, OND leaders, and external stakeholders to disseminate information and updates on scientific and regulatory activities under the purview of the OND Director and Deputy Directors.

In response to the COVID-19 pandemic, OND created the COVID-19 PD Mailbox to respond to inquiries, track new submissions, and facilitate jurisdiction assignments for CDER-regulated products intended to treat COVID-19. ExecOps, with help from ORO, manages the mailbox tracking and responding to requests. ExecOps oversaw the Office of Business Informatics (OBI) development of a dashboard designed to provide real-time data to leadership regarding inquiries, meetings, and pre-IND applications for CDER-regulated COVID-19 product development programs.

PDIMS tracks program related user-fee requirements, responds to internal and external program-related questions, and works to standardize program-related review work across OND to the extent possible. Due to the shift to a virtual work environment with the pandemic, sponsors faced challenges submitting paper submissions, and simultaneously OND's ability to receive paper submissions became difficult. PDIMS worked with OBI to expand the NextGen Portal to accept electronic submissions that were not in the electronic common technical document format, enabling sponsors of research INDs to submit electronically rather than via mailed paper submissions.

In 2020, PDIMS also provided subject matter expertise on expedited programs as part of a CDER initiative to create and publish the [Compilation of CDER New Molecular Entity \(NME\) Drug and New Biologic Approvals Project](#), a high-level public compilation of data on CDER NME and new biologic approvals from 1985 to 2019 in a single user-friendly file.

LTD's top priority in 2020 was to support the NDRP Modernization efforts by providing learning and organization development resources so OND staff could continue to perform their roles efficiently during the ongoing change efforts. LTD supported the IND and Integrated Assessment workstreams to produce over 60 eLearning modules and five new courses, which trained over 1,000 review staff across CDER.

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The ExecOps team triaged over 2,400 COVID-19 related inquiries in the COVID-19 Product Development (PD) Mailbox!

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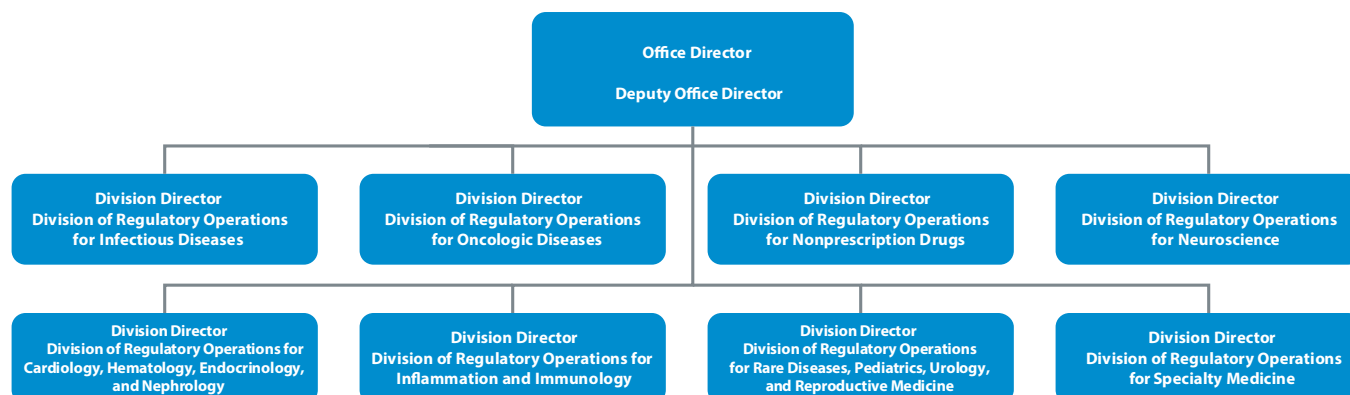
LTD enrolled 51 leaders, including Team Leaders, in OND's Leadership Development Coaching Program in 2020!

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In March 2020, LTD shifted training and education programs to a virtual delivery format due to COVID-19. LTD staff increased communication and used multiple platforms to reach attendees while continuously improving different processes to better collect feedback, questions, and requests. LTD's Regulatory Project Management (RPM) Academy saw a 300% increase in attendance after moving to a virtual platform, with 2,742 attendees as of June 2020. LTD's Introduction to Clinical Review Series also saw enrollment increase to over 1,200 participants.

OPO has been collaborative and adaptive in 2020, making necessary adjustments to support OND during a pandemic and in a virtual environment.

## Office of Regulatory Operations (ORO)



The OND reorganization created ORO to centralize RPM activities and provide more consistent, cohesive, and strategic regulatory expertise and project management support to OND's clinical offices. Before the reorganization, OND placed RPMs within clinical divisions. Now, ORO consists of eight Divisions of Regulatory Operations, which are comprised of groups of RPMs that align with each of the OND clinical divisions. After OND's reorganization in March 2020, ORO's main objective was to stand-up the new office and establish its leadership positions. Despite transitioning to a fully virtual work environment shortly after its formation, ORO successfully achieved its hiring goals, with selections made for most of ORO's leadership positions.

Like many offices across FDA, ORO had to quickly adapt to the additional workload caused by COVID-19 drug development. One of ORO's biggest accomplishments in 2020 was creating an updated and modernized system to handle incoming COVID-19 requests and proposals. ORO, in collaboration with OPO, modified existing regulatory operations and IT systems to accommodate thousands of COVID-19 external inquiries and submissions. Additionally, after OPO established a mailbox as the central point of entry for COVID-19 related inquiries for OND, ORO designed a triage process for ORO RPMs and OND clinical staff to evaluate inquiries and route them appropriately within OND. The triaging of the enormous amount of inquiries helped reduce the burden on divisions that were hit particularly hard and found themselves inundated by COVID-19 related requests.

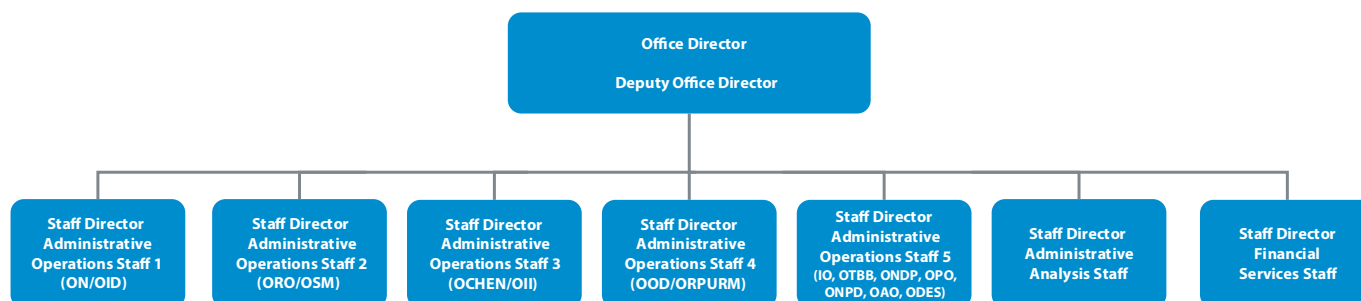
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ORO staff developed the triage process for evaluating incoming COVID-19 inquiries and routing them to the appropriate OND office and division.

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Along with its COVID-19 specific activities, ORO also helped OND offices transition to the new virtual work environment. ORO staff, particularly RPMs, provided platforms to assist the review teams in conducting internal and industry meetings, completed review work, and provided timely, much-needed feedback on industry proposals for COVID-related issues and applications. Even during the COVID-19 national emergency, ORO RPMs gave regulatory presentations at the [Regulatory Education for Industry \(REdI\) Annual Conference](#), CDER Small Business & Industry Assistance session, the National Regulatory Commission, and the [Regulatory Affairs Professional Society Conference](#). These presentations helped stakeholders better understand the safety and efficacy standards required for drugs to be safely marketed to people. Lastly, ORO collaborated with other offices and Centers to clear over a dozen COVID-19 guidances in 2020 to provide industry with information on how FDA was managing COVID-19 and the processes surrounding drug applications.

## Office of Administrative Operations (OAO)



During the OND reorganization, the Program Management and Analysis Staff in OND was converted to OAO. OAO provides the highest level of customer-focused administrative management services to OND staff and is the administrative arm of OND supporting human capital, budget, travel, and time and attendance activities. OAO is comprised of seven staff responsible for all administrative management needs for assigned, dedicated OND offices. OAO's main priorities in 2020 were recruitment and budget restructuring/streamlining due to OND's reorganization.

OAO facilitated the recruitment and hiring efforts for newly created leadership positions across OND. As the OAO team had to do this while rebuilding relationships with hiring managers, the office collaborated extensively with their human capital partners, CDER's Office of Management (OM) and Office of Talent Solutions, to ensure the hiring process was more efficient and cohesive as compared to before the reorganization.

OAO also developed dedicated Human Capital resources focused on recruitment and hiring during the transition to a virtual onboarding process. OAO worked diligently to make the hiring process smoother by collaborating and partnering with internal stakeholders to create more efficient and streamlined onboarding processes.

As part of the OND reorganization, OAO established the Financial Services Staff to provide oversight and management for planning and execution of the office budget portfolio. The dedicated resources of this staff ensure that funding is available to support the review of medical products.

In OND's virtual environment, OAO staff diligently provided equipment, tools, and resources to staff, including volunteering to come into the office to ship items to employee's duty stations. This is a testament to OAO's commitment to ensure all OND staff can continue to perform their work at the highest level.

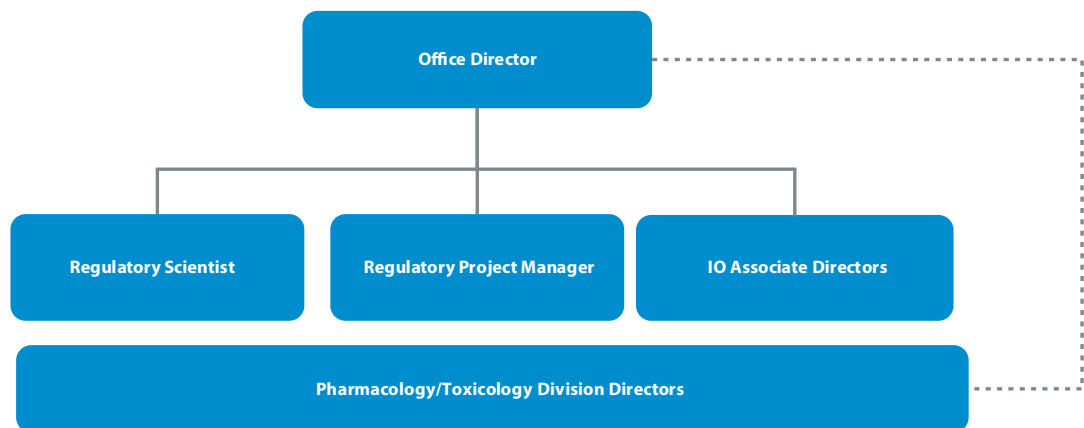
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OAO is excited about the addition of their new Office Director, whose experience and strategic vision supports OAO's goals to enhance communication, motivate staff, and meet the administrative needs and mission of OND.

The transition of OAO's new supervisors into their assigned staff is also notable. They enabled their staff to thrive by utilizing leadership and management practices that empower direct reports to work collaboratively and in dialogue with each other and across the team. OAO has created a "We've Got Your Back" culture in conjunction with other office supervisors.

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## Pharmacology/Toxicology (Pharm/Tox)



Pharm/Tox plays a vital role in drug development to ensure the safety of new drugs developed in OND. Pharm/Tox reviewers evaluate nonclinical data for new drugs to aid in the selection of safe starting and maximum doses for “first in human” clinical trials. Reviewers also look for potential toxicities to monitor in clinical trials and assess toxicities not addressed in trials.

OND’s Pharm/Tox organization has IO staff, as well as six divisions located across, and supporting, the OND clinical review offices: the Division of Pharm/Tox for Infectious Diseases (DPT-ID), part of OI; the Division of Pharm/Tox for Immunology and Inflammation (DPT-II), part of OII; the Division of Pharm/Tox for Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (DPT-RPURM), part of ORPURM but also supports OSM; the Division of Pharm/Tox for Cardiology, Hematology, Endocrinology and Nephrology (DPT-CHEN), part of OCHEN; the Division of Pharm/Tox for Neuroscience (DPT-N), part of ON; and the Division of Hematology Oncology Toxicology (DHOT), part of OOD. ONPD continues to be supported by the Pharm/Tox Associate Director and their staff.

Pharm/Tox established a Senior Leadership Team (PT-SLT) to coordinate between the policy-setting group (Pharm/Tox IO) and review divisions (PT-Division Directors). The PT-SLT adopted and shared best practices documents for consistent work processes across the staff. The PT-SLT was instrumental in the planning and implementation of the structural reorganization of the Pharm/Tox staff as part of the NDRP Modernization effort. While most Pharm/Tox staff continued working with the same clinical divisions in the same therapeutic areas, others paired with new clinical divisions.



Most of the DPT-ID's work this year shifted to COVID-19 beginning in March 2020. The team focused on managing the larger workload and collaborated across the Pharm/Tox divisions to solicit volunteer reviewers to assist with the influx of COVID-19 pre-INDs and INDs. Staff had to manage quick turnaround times for the COVID-19 work and collaborated more internally to receive input from other Pharm/Tox divisions on drug applications.

In 2020, DPT-II was heavily involved in the nonclinical review of potential COVID-19 therapeutics, specifically the staff supporting Pulmonology, Critical Care, and Rheumatology. In particular, the division provided expertise and safety assessments of novel products proposed for the inhalation route of administration in COVID-19 patients.

DPT-RPURN's priority in 2020 was to bring together the staff from several new clinical indications included in ORPURN, including ophthalmology, medical genetics, and rare diseases. DPT-RPURN successfully collaborated with ORPURN's Division of Pediatrics and Maternal Health (DPMH) to set up a workshop focused on drug development and the inclusion of pregnant women in clinical trials. This workshop is an essential step towards increasing clinical research in pregnant women to develop a clearer scientific understanding of the risks and benefits associated with the use of medications during pregnancy.

DPT-CHEN focused on establishing staff and structure in the new division. The immediate public health crises of COVID-19 greatly impacted the nonclinical review staff that serve the Division of Cardiology and Nephrology (DCN) and Division of Non-Malignant Hematology (DNH). These two clinical divisions have overseen a multitude of COVID-19-associated pre-INDs and INDs. Their collaboration across disciplines, adaptability, and dedication to assessing the nonclinical information of these drug applications helped support the transition to clinical trials.

DPT-N's main priority this year was standing up the new division and recruiting new reviewers, a challenge in the remote working environment. In addition to this and its drug review support, DPT-N supported ON in communications regarding the U.S. opioid crisis and on safer drug labeling protocols for opioids. DPT-N was also involved in individualized therapies in neurology, collaborating with National Center for Toxicological Research (NCTR) on research to better characterize the toxicity of ketamine in the adolescent brain using animal models.

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The Pharm/Tox IO staff played an important role in OND's work on hand sanitizer during the COVID-19 pandemic, which saw shortages in hand sanitizer ingredients and safety-related issues. Several rapidly developed guidances outlined temporary policies on hand sanitizers and included information on acceptable impurity and excipient levels, which required significant Pharm/Tox input. Volunteers from other divisions helped review the safety of alcohol denaturants. Staff spent significant overtime addressing hand sanitizer issues.

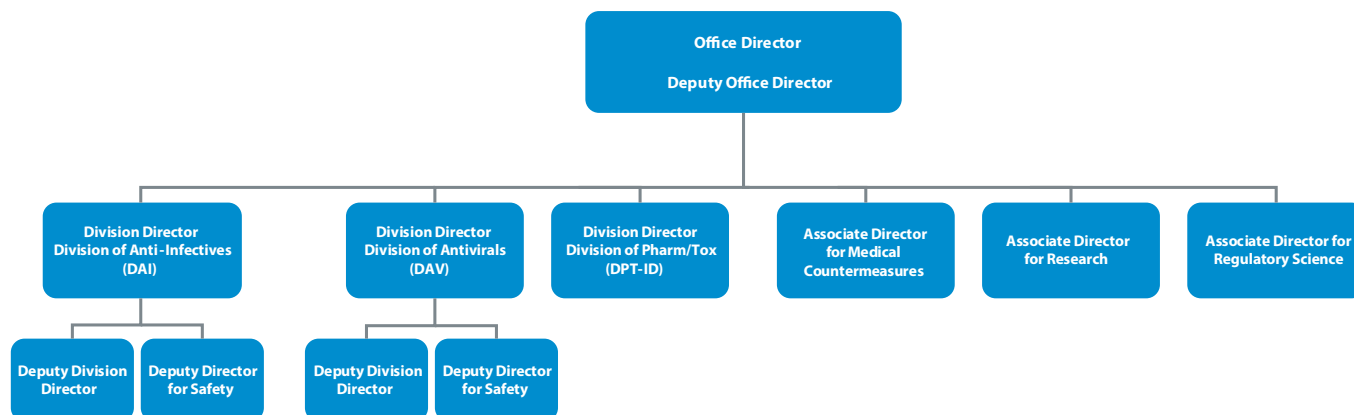
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DHOT reviews the nonclinical pharmacology and toxicology aspects of therapies to treat patients with cancer. Due to the serious and life-threatening nature of cancer, DHOT often operates under expedited timelines for the review of many NDAs and BLAs. For this reason, the oncology review team strives to approve products as quickly as possible to get products to patients without sacrificing review quality. This quick Oncology Office self-imposed turnaround means review timelines are three to four months rather than the typical PDUFA timeline of ten months. In addition to supporting the review of therapies to treat patients with cancer, DHOT continued its participation in projects at the Oncology Center of Excellence (OCE). DHOT helped review submissions as part of OCE's Project Orbis, which provides a framework for concurrent submission and review of oncology products among international partners.

ONPD Pharm/Tox staff helped implement the Over-the-Counter (OTC) monograph reform, which was included in the passage of the [Coronavirus Aid, Relief, and Economic Security \(CARES\) Act](#), a COVID-19 economic stimulus package that was signed into law in March 2020. ONPD Pharm/Tox staff also addressed safety issues related to inhaled nicotine delivery devices ('vaping'), including finalizing an industry guidance on this topic.

## Office of Infectious Diseases (OID)



The OND reorganization did not significantly impact OID and its three review divisions: the Division of Anti-Infectives (DAI), DAV, and DPT-ID. OID oversees the development, review, and regulation of new antiviral and anti-infective drugs. Throughout 2020, OID was heavily engaged in the CDER response to the COVID-19 pandemic while continuing to focus on addressing the ongoing challenge of antimicrobial drug resistance and the unmet need for new antiviral and anti-infective drugs.

COVID-19 has been OID's number one priority since February 2020, as the novel coronavirus, SARS-CoV-2, caused a global pandemic and CDER turned its attention to supporting the development of COVID-19 therapeutics. OID received over 310 pre-IND and IND submissions related to development of therapeutics for COVID-19, and the office focused on facilitating drug development via providing advice to sponsors, developing guidances, and staffing the OND "triage team" to facilitate complete pre-IND packages. OID played a critical role in the review of EUAs for hydroxychloroquine/chloroquine, remdesivir, bamlanivimab, and casirivimab/imdevimab. Based on OID's review, FDA granted an EUA for remdesivir in May 2020. In October 2020, OID approved an NDA for remdesivir for use in adult and pediatric patients for the treatment of COVID-19 requiring hospitalization, paving the way for remdesivir to become the first FDA-approved treatment for COVID-19. On November 9 and 21, 2020, FDA authorized [EUAs](#) for bamlanivimab and casirivimab/imdevimab, monoclonal antibodies (mAbs) used to treat mild to moderate COVID-19 in adults and pediatric patients with positive results using direct SARS-CoV-2 viral testing.

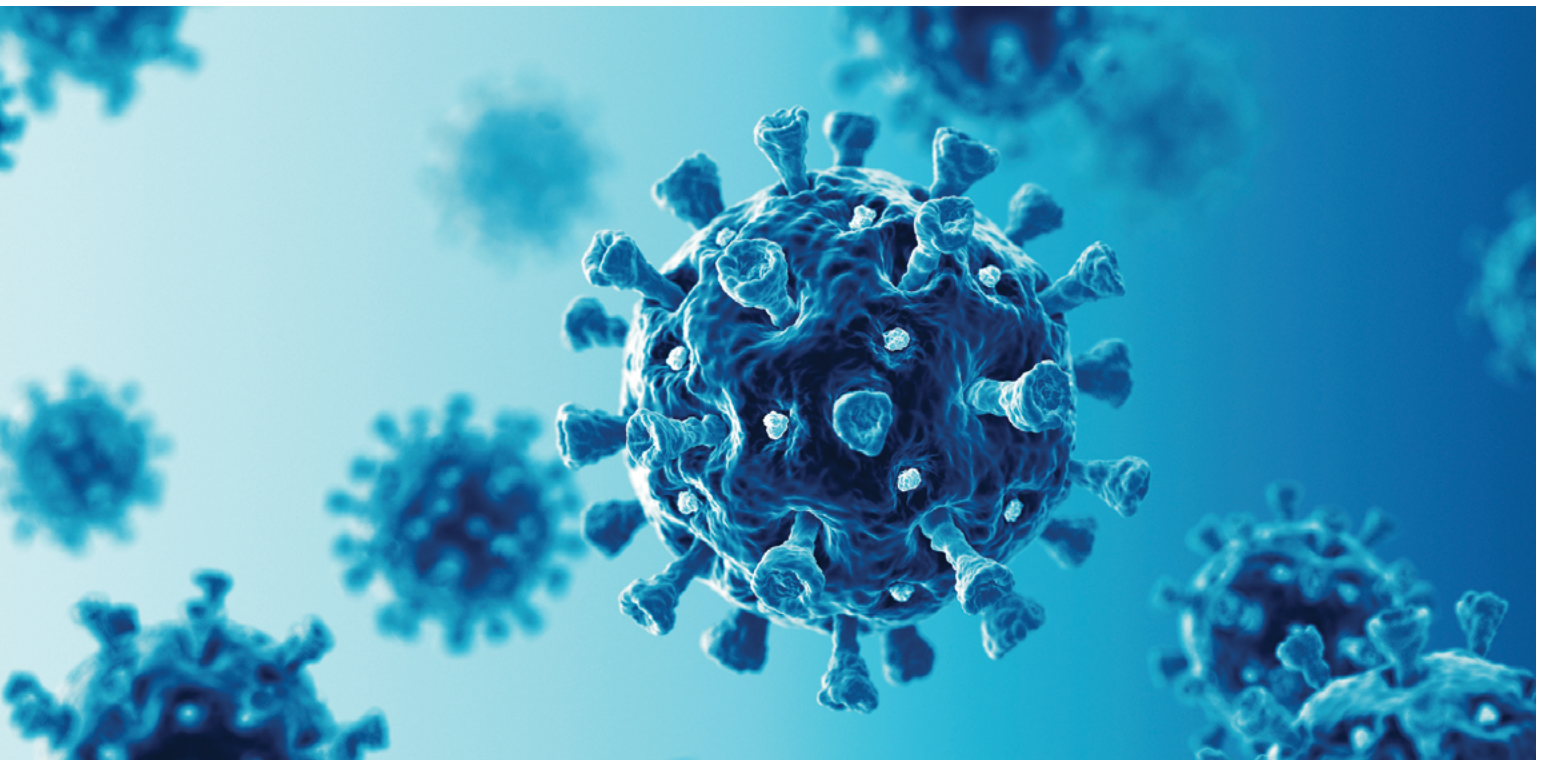
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OID received approximately 424 pre-IND and IND applications for both COVID and non-COVID submissions in calendar year 2020!

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OID also took part in the ACTIV partnership to investigate COVID-19 therapeutics and prophylaxis. More specifically, OID reviewed inpatient and outpatient platform trials for mAbs and provided advice on the development of several protocols under pre-INDs in a short turnaround time. OID continues to provide ongoing advice regarding the implementation of platform trials.

Beyond COVID-19 work in 2020, DAV focused extensively on human immunodeficiency virus (HIV)-related drug reviews, approvals, and clinical trial designs. In July 2020, after completing an expedited Priority Review, DAV approved Rukobia (fostemsavir), a breakthrough therapy designated as a first-in-class treatment for adults with multidrug-resistant HIV infections. DAV also tentatively approved HIV treatments for use under the President's Emergency Plan for AIDS Relief, despite being hampered by pandemic-related travel restrictions for manufacturing site inspections. In collaboration with the [Forum for Collaborative Research](#), DAV is developing a novel approach to calculate HIV incidence in various countries and regions to use as an external control when traditional prevention trial designs are not feasible. In addition to its HIV-related review work, DAV also approved a priority review of [Inmazeb](#) (atoltivimab, maftivimab, and odesivimab-ebgn) to become the first FDA-approved treatment for the Ebola virus infection in adult and pediatric patients. This approved treatment for the Ebola virus marks a critical milestone in public health preparedness and response.

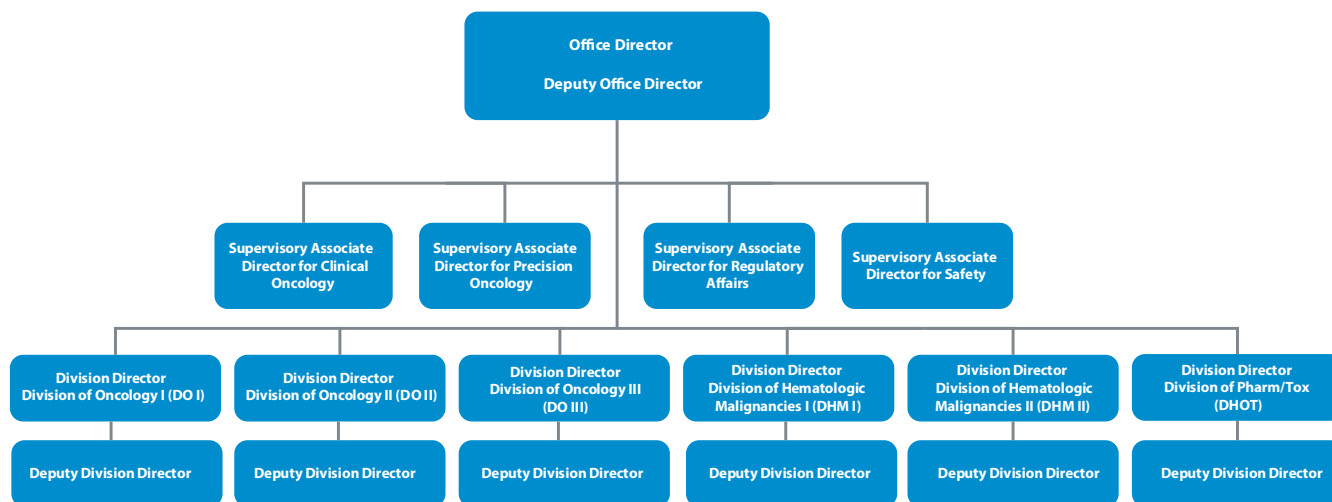


DAI reviewed several notable NDAs in 2020. In May 2020, FDA granted full approval and tentative approval, respectively, to two priority review NDAs for artesunate injection for the treatment of severe malaria in adult and pediatric patients. At the time of the approvals, there were no marketed drugs to treat severe malaria in the U.S. In August 2020, FDA approved a priority review NDA for Lampit (nifurtimox) for the treatment of Chagas disease in pediatric patients from birth to less than 18 years of age. DAI also completed two priority reviews of supplemental NDAs (sNDAs) that provided important treatment options for the treatment of hospital acquired and ventilator-associated bacterial pneumonia. One sNDA was for Recarbrio (imipenem-cilastatin/relebactam) and the other was for Fetroja (cefiderocol). Approval of the Fetroja sNDA marked the first time that dosing information for patients on continuous renal replacement therapy was included in antibacterial drug labeling.

DAI worked with the Center for Veterinary Medicine to draft a revised process for ranking antimicrobials according to their relative importance in human medicine. In November 2020, the division held a public meeting to obtain input on a [concept paper](#) outlining the aforementioned potential revised process. DAI continued its work on optimizing the design of clinical trials in infections with unmet medical need, including via a March 2020 workshop that covered progress and challenges in the development of various animal models for serious infections and identified potential future research priorities in this area. The workshop summary has been accepted for publication in the *Journal of Antimicrobial Chemotherapy*.

OID started the year redirecting their efforts toward COVID-19 and ended it with the approval of the first treatment for COVID-19. In addition to being at the forefront of CDER's COVID-19 response, OID continued efforts to address antimicrobial drug resistance, focused on optimizing clinical trials for infections with unmet need, reviewed and approved notable new therapeutics for HIV infection, Ebola virus, nosocomial pneumonia, malaria, and Chagas Disease.

## Office of Oncologic Diseases (OOD)



OOD oversees development, approval, and regulation of drug and biologic treatments for cancer and hematologic malignancies. OOD includes five clinical divisions organized by cancer type plus one nonclinical toxicology division. OOD is responsible for making sure that drugs and biologics to treat cancer are safe and effective for the U.S. public. OOD staff collaborate with specialists in OCE and CBER, Center for Devices and Radiological Health, and CDER science disciplines to independently review data on new treatments for cancer. Scientists within OOD work hard to incorporate innovations in pharmacogenomics, bioinformatics, and clinical trial design into the drug development process to accelerate the introduction of new cancer treatments for patients.

Despite the COVID-19 pandemic, OOD continued to review new oncologic drugs and therapies, worked to maintain virtual connections with all cancer community stakeholders, and reached out to patient advocacy groups to ensure that cancer patients could access their therapies during the pandemic. OOD expedited approvals related to new dosing regimen(s) for patients receiving cancer treatment during the COVID-19 pandemic where both patients and providers wanted to reduce interaction in the health care setting to reduce transmission of the coronavirus. These included approvals of the oral drug Ayvakit (avapritinib), a new dosing regimen for Keytruda (pembrolizumab), a subcutaneous formulation of Phesgo (pertuzumab/trastuzumab), and many others.

OOD also successfully transitioned to a remote work environment, with staff check-ins, organized breaks at the virtual “water cooler,” and phone conversations to help the team stay connected. OOD assigned mentors within and across different teams to interact with new hires, and OOD staff developed an educational curriculum with a virtual lecture series discussing key regulatory topics from experienced reviewers and leadership to keep employees informed and engaged with OOD’s mission.

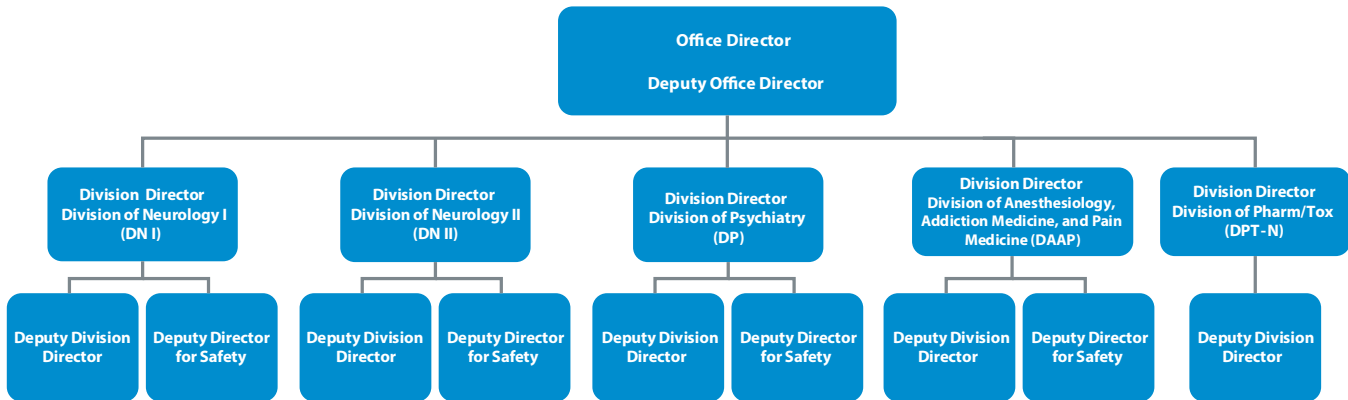
Despite the heavy workloads and additional stress of COVID-19, OOD staff continued to fulfill and exceed their research and cancer drug review mission.

Project Avatar, a project encouraging certain OOD meetings to be conducted 100% virtually on webcam, was initiated in January 2020 to allow all OOD staff to virtually participate in meetings, which ensured a seamless transition into the full-time telework environment during the COVID-19 pandemic.



## OOD Leadership Meeting

## Office of Neuroscience (ON)



ON seeks to protect and promote the public health in the neuroscience space and oversees five review divisions that regulate and review INDs and marketing applications for drug and biologic products for a spectrum of diseases and conditions, including, among many others, neurodegenerative movement, neuromuscular disorders, seizures, epilepsies, migraines, traumatic brain injury, stroke, neuroimmunologic disorders, bipolar disorder, schizophrenia, major depressive disorder, attention deficit hyperactivity disorder, obsessive-compulsive disorder, post-traumatic stress disorder, anxiety disorders, autism spectrum disorder, sleep disorders, acute pain, chronic pain, and addiction, along with surgical anesthetics, sedatives, and neuromuscular-blocking agents. ON comprises of the Division of Neurology 1; the Division of Neurology 2; the Division of Psychiatry; the Division of Anesthesiology, Addiction Medicine, and Pain Medicine; and DPT-N.

As an office newly created from the OND reorganization, ON worked in 2020 to establish the office and fill leadership roles while focusing on advancing drug development. Neuroscience has entered an era of exponential growth, with explosive progress in the understanding of diseases that until recently had no treatment. Innovative treatment modalities, such as antisense drugs and small interfering ribonucleic acids, were pioneered in ON and are becoming more and more widespread. ON's new structure allows the office to "de-silo" staff to provide a more holistic approach to the regulation and review of neuroscience drugs and to leverage expertise across the office so that all divisions benefit from shared knowledge and ensure greater consistency in ON's regulatory approaches.

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"Until last year, there were no FDA-approved treatments for patients with this rare, debilitating and sometimes fatal disease [NMOSD]. Now there are three," said Billy Dunn, M.D., Director of the Office of Neuroscience.

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In 2020, ON approved two major drugs providing groundbreaking treatment for neuromyelitis optica spectrum disorder (NMOSD). NMOSD is a rare, chronic autoimmune disease that affects the optic nerves, spinal cord, and brain stem. ON approved two new therapies for NMOSD: Uplizna (inebilizumab-cdon) and Enspryng (satralizumab-mwge). There are now three effective therapies on the market for this rare disease.

In 2020, ON continued to focus on stakeholder engagement to facilitate and expedite development of a new generation of neuroscience drugs. ON guides the field by engaging with and participating in critical neuroscience scientific gatherings and discussions around the world. ON strives to be a leader in innovation in drug development for neuroscience, embracing and making routine progressive approaches including novel methodologies, quantitative analytics, modeling and simulation, decentralized trials, use of technological advances, and explorations of novel approaches to control/comparator groups — all done rigorously and with attention to developing new therapies for patients as efficiently as possible.

Another high-impact area for ON in 2020 was labeling changes for benzodiazepines and opioid pain medicines. ON added a [Boxed Warning label on benzodiazepines](#) that includes the risks of abuse, misuse, addiction, physical dependence, and withdrawal reactions associated with this class of drugs. In addition to the Boxed Warning update, FDA is requiring other labeling changes and changes to the existing patient Medication Guides for all benzodiazepine products to help educate users about the risks of benzodiazepines. ON also required that labels for opioid pain medicines and medicines used to treat opioid use disorder be updated to indicate that health care professionals may discuss the availability of naloxone with patients as a routine part of prescribing these medications. Naloxone is a medicine that can be administered by individuals with or without medical training to help reduce opioid overdose deaths.

In response to the global pandemic, ON employees volunteered to assist other medical reviewers in OND's pulmonary and antiviral divisions, who had exponentially increased workloads due to COVID-19. ON employees worked closely with the OII's DPACC, exemplifying OND's collaborative spirit.

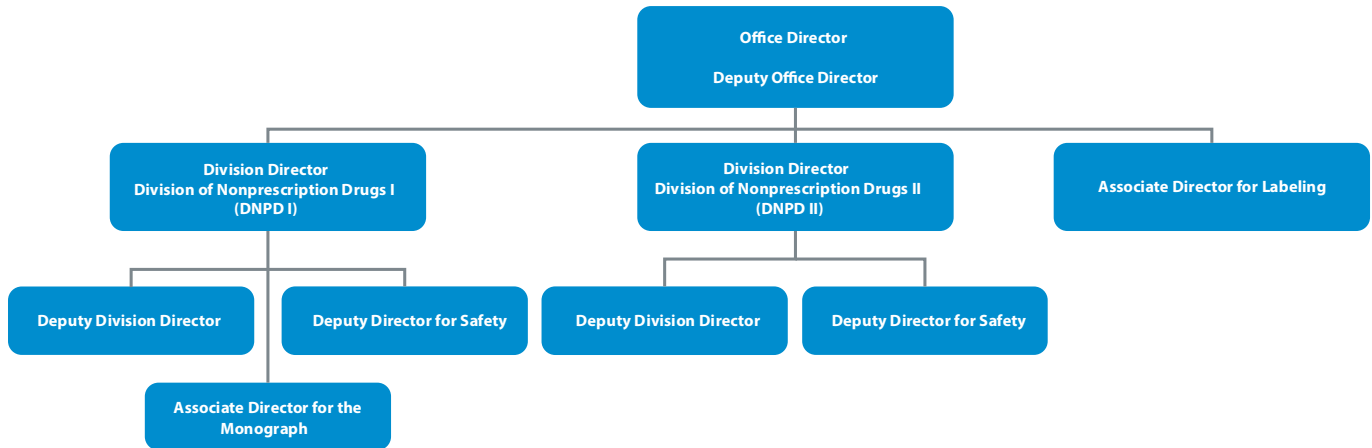
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“Even during this global pandemic, we have continued to prioritize addressing the opioid crisis. Today’s action can help further raise awareness about this potentially life-saving treatment for individuals that may be at greater risk of an overdose and those in the community most likely to observe an overdose. We will use all available tools to address this crisis, and we know efforts to increase access to naloxone have the potential to put an important medicine for combatting opioid overdose and death in the hands of those who need it most — those at increased risk of opioid overdose and their friends and family.”

— FDA Commissioner Stephen Hahn.

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## Office of Nonprescription Drugs (ONPD)



ONPD leads the development, review, and regulation of nonprescription drugs, which include more than 100,000 marketed drugs. ONPD has two review divisions: the Divisions of Nonprescription Drugs 1 and 2.

ONPD is unique compared to other clinical offices in OND, as there are two pathways to bring a nonprescription drug to market: the NDA process and the OTC Drug Review (or OTC monograph) process. Unlike NDAs for prescription drugs, FDA may require a sponsor of a nonprescription NDA to conduct consumer behavior studies to demonstrate that consumers can use the product safely and effectively without the supervision of a health care provider.

The OTC monograph system is CDER's largest regulatory space, covering approximately 800 different active ingredients with more than 1,400 uses. Under the system, drugs may be marketed without an approved application if the drugs comply with statutory requirements and applicable conditions in an OTC monograph. An OTC monograph is a "rule book" of conditions for each therapeutic category, describing the active ingredients, uses, doses, route of administration, labeling, and testing for an OTC drug to be considered generally recognized as safe and effective.

In 2020, ONPD staff focused on implementing new statutory provisions that reformed and modernized the OTC monograph process. The provisions were part of the CARES Act and aim to create a more streamlined administrative process to establish or revise OTC monographs that is intended to spur OTC drug innovations that promote consumer choice and allow FDA to rapidly address

safety issues. ONPD staff analyzed and interpreted the many legal and regulatory questions, conducted outreach to external stakeholders, drafted guidance documents, converted existing OTC monographs into final orders, and put the necessary infrastructure in place to implement the OTC monograph reform provisions.

The CARES Act also provided FDA the authority to assess and collect user fees dedicated to OTC monograph drug activities. Prior to the passage of the CARES Act, OTC monograph drugs were the only category of FDA-regulated drug products not funded by user fees. ONPD is working jointly with CDER's OM to implement the Over-the-Counter Monograph User Fee Act (OMUFA). FDA anticipates that OMUFA will provide additional resources to ultimately help provide the public access to innovative OTC monograph drugs.

With the increase in hand sanitizer demand due to the outbreak of COVID-19, ONPD staff heavily engaged in work across several areas related to hand sanitizers to help meet the increased demand and ensure hand sanitizer safety. The office developed and contributed to several [guidances](#) with multiple updates related to [manufacturing](#) and [compounding](#) hand sanitizers. With the rise in reports of serious adverse events such as blindness and death, ONPD supported the recall of hand sanitizer products contaminated with methanol and 1-propranolol and provided numerous health hazard evaluations to support recalls for various hand sanitizer safety issues. ONPD also worked to educate consumers on proper use of hand sanitizers. Increased incidence of accidental ingestion of hand sanitizer by children led the office to publish its [findings](#) in collaboration with OSE and to [warn consumers of hand sanitizer packaged in food and drink containers that could be attractive to children](#). ONPD also participated in national and international stakeholder calls on methanol contamination in hand sanitizer. Due to the impact of COVID-19, hand sanitizer safety issues will continue to be a priority for ONPD in 2021.

In addition to their normal review responsibilities, ONPD staff managed a heavy COVID-19 workload as the primary review division for COVID-19 therapeutics of all nonprescription drugs, as well as all applications and meeting requests for drugs (prescription and nonprescription) administered via a topical or nasal route.

In 2020, ONPD also collaborated with multiple internal and external partners to publish a model Drug Facts label (DFL) for OTC naloxone. FDA continues to combat the opioid crisis and expand the use of naloxone, and industry had expressed concern that the need to do consumer studies for OTC naloxone was a barrier to the potential development and regulatory approval. This model DFL underwent independent testing to determine whether consumers could understand

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ONPD co-led two separate FDA negotiating teams through several years of policy and user fee discussions with industry on both elements of the administrative procedures included in the monograph reform and the monograph user fee system. The user fee negotiations with industry were the first ever with this manufacturing segment.

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ONPD staff prioritized work on hand sanitizer safety with the outbreak of COVID-19. ONPD supported the recall of hand sanitizer products that were causing serious injuries to the general public.

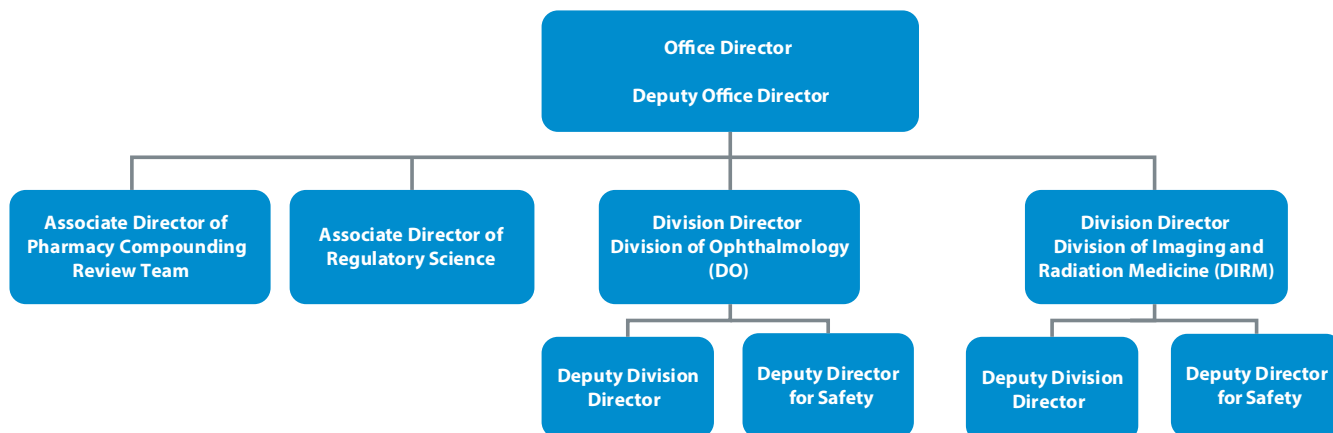
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the key statements on the label for safe and effective use in an emergency. The consumers in the study demonstrated sufficient understanding of six of the eight components of the instructions in the DFL for naloxone use and were near target on the remaining two components. A separate FDA review group found that the model DFL was adequate for use in the development of a naloxone product intended for OTC sales. ONPD hopes availability of this model DFL will shorten the development time for OTC naloxone, which will lead to wider public access. ONPD published a [study](#) on the model DFL in the *New England Journal of Medicine*.

Sunscreen safety was another important focus for ONPD in 2020. To support sunscreen safety, ONPD staff conducted a number of key studies, including one testing whether various sunscreen ingredients can be absorbed through the skin and into the body. The study showed that even a single application of sunscreen to the skin resulted in measurable blood levels of the active ingredient for all the tested active ingredients and formulations. ONPD noted these study results do not mean the ingredients are unsafe, but they do call for further industry testing to determine the safety and effects of systemic exposure to sunscreen ingredients. FDA published the [study](#) in the *Journal of the American Medical Association*.



## Office of Specialty Medicine (OSM)



As one of the last offices to be restructured as part of the OND reorganization, OSM retained the Division of Imaging and Radiation Medicine (DIRM) and absorbed the new Division of Ophthalmology (DO). OSM continued to emphasize external stakeholder engagement, develop guidances for conditions where there is a significant interest in drug development, and utilize innovative solutions for scientific and regulatory issues that arise during drug development and in the review of drug applications.

In addition to DIRM and DO, OSM focused on standing up an OND pharmacy compounding review team in 2020. This multi-disciplinary team, headed by the Associate Director of Pharmacy Compounding, leads the review of bulk drug substances nominated for inclusion on the FDA 503A and 503B compounding list.

While 2020 was a challenging year, OSM continued its strong focus on external stakeholder engagement. DO staff attended and spoke at 11 external talks and panel engagements, including meetings with the American Academy of Ophthalmology, American Glaucoma Society, American Society of Cataract and Refractive Surgeons, and American Society of Retinal Surgeons. DIRM continued to regularly collaborate with stakeholders to spur the development of radiation medicine products that lack commercial incentives, allow access under expanded access protocols, show a regulatory path to approval, and invite further commercial development. DIRM engaged other government agencies (e.g. Nuclear Regulatory Commission), academic investigators, and professional societies (e.g. Society of Nuclear Medicine and Molecular Imaging) and collaborated with academic investigators and professional organizations to develop a new targeted class of Positron Emission

Tomography (PET) drugs for unmet medical needs. These efforts aimed to increase the availability of radionuclides, such as gallium-68, needed to produce the imaging drugs and to develop and promote the adoption of standard clinical trial protocols for the academic studies that provided the evidence of safety and effectiveness of the new drugs.

To that end, DIRM made significant advancements in PET imaging agents in 2020. In May 2020, FDA approved Tauvid (flortaucipir F18) for intravenous injection, the first drug used to help image a distinctive characteristic of Alzheimer's disease in the brain called tau pathology. This product estimates the density and distribution of aggregated tau neurofibrillary tangles, a primary marker of Alzheimer's disease. In December, FDA approved a PET drug for detection and localization of prostate cancer (68Ga-PSMA-11, no trade name). The performance of this agent offers a meaningful advance for imaging patients with primary and recurrent prostatic cancer.

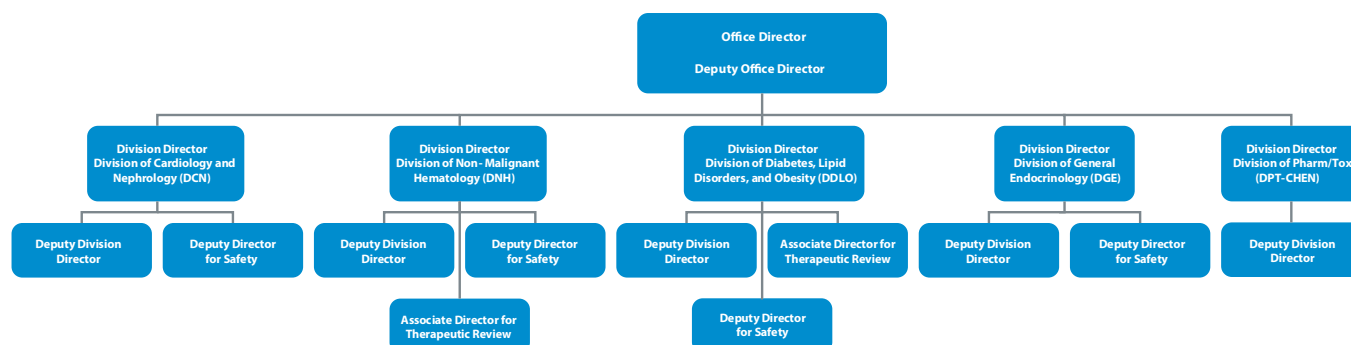
For ophthalmology, FDA approved Tepezza (teprotumumab-trbw) to treat adults with thyroid eye disease — the first drug approved for the rare condition in which the muscles and fatty tissues behind the eye become inflamed, causing the eyes to be pushed forward and bulge outwards. Tepezza has the potential to alter the course of the disease, potentially sparing patients from needing multiple invasive surgeries by providing an alternative, non-surgical treatment option.

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Through their collaboration and engagement with clinical experts, OSM approved four novel diagnostic and therapeutic drugs and published 14 articles in medical literature in calendar year 2020.

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## Office of Cardiology, Hematology, Endocrinology, and Nephrology (OCHEN)



OCHEN oversees the development, review, and regulation of applications for drug and biologic products reviewed across cardiovascular, metabolic, renal, endocrine, bone, and hematologic diseases and plays a significant role in advancing public health in the U.S., regulating drugs critical to more than a third of the U.S. population. OCHEN was formed in late 2019 as part of the OND reorganization and consists of five review divisions: DCN, the Division of Diabetes, Lipid Disorders and Obesity (DDLO), the Division of General Endocrinology (DGE), DNH, and DPT-CHEN.

OCHEN's first year goals in 2020 included staffing key supervisory positions within the divisions and integrating pharmacology and toxicology expertise across the divisions. The office was successful in retaining and hiring talented supervisors for most leadership positions, but because the personnel originated from four different prior OND offices, an ongoing OCHEN goal is to integrate leadership approaches across the divisions to reach a consistent set of practices across the new office. OCHEN made good early progress towards this goal in 2020, with a considerable amount of "cross-pollination" that has helped standardize the office's regulatory approaches.

DCN was instrumental in the issuance of an EUA to permit the emergency use of the unapproved product, Fresenius MultiBic solution, for use in hemofiltration and as a dialysis solution in patients requiring renal replacement therapy. DNH's involvement in the COVID-19 pandemic also included the review of multiple INDs for antiplatelet agents, anticoagulants, and agents that inhibit complement.

OCHEN staff participated actively in outreach, guidance writing, public workshops, and meetings throughout 2020. One of OCHEN's major accomplishments in 2020 was an extensive update to the [draft guidance](#),

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OCHEN played an important role in the issuance of two COVID-19-related EUAs, both intended to avert shortages of solutions used in renal replacement therapies.

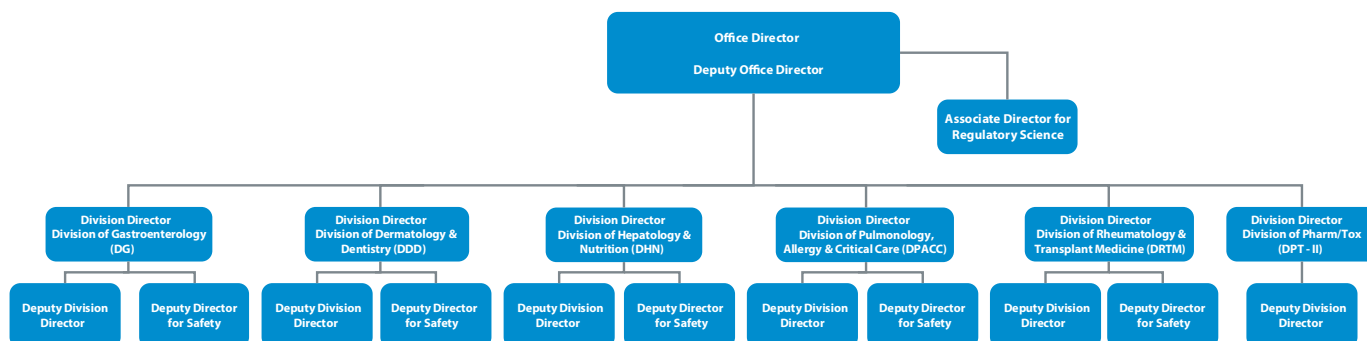
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which revised FDA's prior recommendations to propose an updated approach to evaluate safety and improve glycemic control. OCHEN also made important contributions to the [draft guidance](#) on how nonclinical assays can be used in an integrated risk assessment before first-in-human studies or in later phases of clinical development to reduce the number of 'Thorough QT' clinical studies.

OCHEN's review work has a considerable impact on public health. Their work regulates drugs and biologics for major conditions affecting morbidity and mortality in the U.S., including cardiovascular disease, chronic kidney disease, diabetes, lipid abnormalities, hematologic diseases, and osteoporosis. Despite being a newly formed office, OCHEN staff were able to work together to fulfill their mission amidst the COVID-19 pandemic and transition to a fully remote work environment.



## Office of Immunology and Inflammation (OII)



OII was newly created after the OND reorganization to align disease areas more closely and bring together the divisions that work on autoimmune diseases and frequently regulate the same products for different indications. OII consists of six review divisions: the Division of Dermatology and Dentistry (DDD), DPACC, the Division of Gastroenterology (DG), the Division of Hepatology and Nutrition (DHN), DRTM, and DPT-II.

In 2020, DDD engaged stakeholder groups through a Critical Path Initiative Meeting to discuss wound treatment and novel endpoints. DDD is continuing to focus on wound treatment as part of the recently launched Science Strategies pilot, which is an approach for disease-specific strategic planning within the Office of Special Programs. A noteworthy approval from DDD in May 2020 was the approval of Dupixent (dupilumab) for children ages six to 11 with moderate-to-severe atopic dermatitis. There are currently no other systemic therapies approved for this indication in this age group.

DPACC received and reviewed over 200 COVID-19 related applications on top of its normal workload over the course of 2020. DPACC completed four noteworthy drug reviews that led to a new approval or expanded patient population. The division completed a priority review of Ofev (nintedanib) as the first treatment for a group of progressive interstitial lung diseases, which FDA approved in March 2020. DPACC also reviewed Orladeyo (berotralstat), an NME for the prevention and treatment of hereditary angioedema attacks and Trelegy Ellipta (fluticasone furoate/umeclidinium/vilanterol), the first triple inhalation therapy product approved for the treatment of asthma in adult patients. Additionally, DPACC expanded the use of Kalydeco (ivacaftor), a treatment for cystic fibrosis, to patients as young as four months of age. Besides these reviews, DPACC also added a Boxed Warning label on serious mental health side effects for the asthma and allergy drug Singulair (montelukast sodium).

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OII reviewed over 265 COVID-19-related applications (a combination of all its divisions hard work)!

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In September 2020, DPACC published a disease progression model of longitudinal lung function decline in idiopathic pulmonary fibrosis (IPF) patients based on the clinical programs for the first two FDA-approved IPF drugs, Esbriet (pirfenidone) and Ofev (nintedanib). Under an RSR-funded project, DPACC developed the model to characterize the observed variability in lung function decline and its decrease in decline after treatment to determine prognostic factors that may be used to enrich IPF studies with patients who are more likely to respond to treatment, potentially enabling IPF studies with smaller study populations or shorter durations.

DG approved several noteworthy therapies in 2020, including Stelara (ustekinumab) to treat patients with ulcerative colitis, and Gimoti (metoclopramide) to treat patients with diabetic gastroparesis. The Gimoti approval was significant, as it is a unique formulation of an existing product that now allows patients to be treated via a nasal administration. DHN maintained a heavy workload reviewing investigational drugs targeting the treatment of NASH and rare pediatric liver diseases and reviewed over three dozen pre-IND packages for nutrition and other drugs proposed for the treatment and/or prevention of COVID-19. DHN is also active in partnerships with academic organizations involved with the pre-marketing and post-marketing evaluations of drug-induced liver injury.

DHN reviewers participated in working groups with the CDER's BQP to assist in qualification efforts for DDTs used in NASH. BQP works with external stakeholders to develop biomarkers as DDTs.

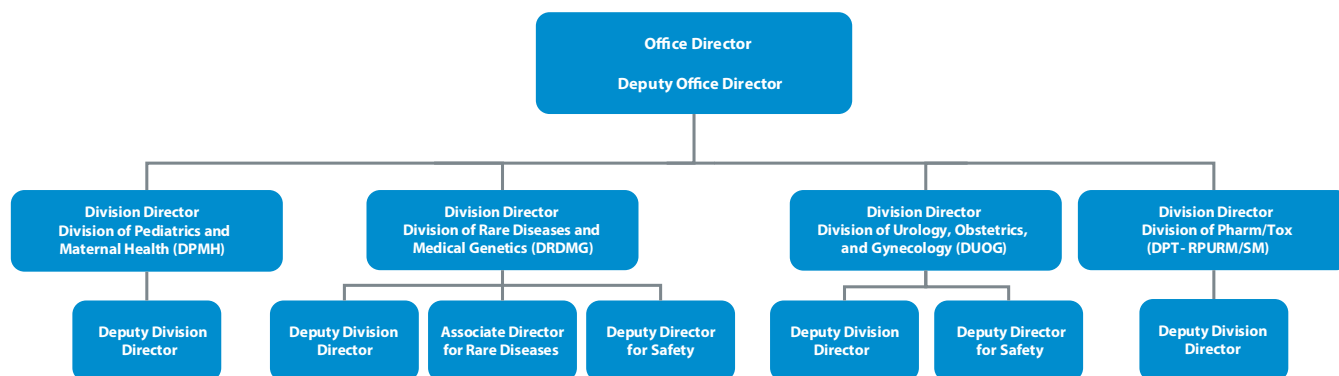
DRTM had to shift priorities to respond to the influx of COVID-19 related applications given the interest in treating COVID-19 patients with medications commonly used for rheumatological conditions. The division, which was formed by combining the rheumatology and solid organ transplant review groups from two former divisions, reviewed over 65 pre-IND and IND applications for immunomodulators and immunosuppressant therapies for COVID-19. DRTM also reviewed data from completed COVID-19 studies, including data submitted to support an EUA for Olumiant (baricitinib). In 2020, DRTM had several notable non-COVID actions, including two new approvals for polyarticular juvenile idiopathic arthritis, one of which was a first-in class, Xeljanz (tofacitinib); the first approval for pediatric psoriatic arthritis, Simponi Aria (golimumab); and the first approval for adult-onset Still's disease, Ilaris (canakinumab).

In collaboration with statistical and clinical pharmacology review teams, DRTM published four peer-reviewed articles in 2020, submitted two manuscripts for review, and presented one scientific poster at the American College of Rheumatology (ACR) Convergence 2020. DRTM

actively engaged with external stakeholders. In October 2020, DRTM hosted a virtual PFDD meeting for systemic sclerosis to obtain patient perspective to inform clinical development in this high unmet medical need area. In November 2020, the division presented at three sessions during the ACR Convergence 2020. DRTM continues to be involved with the Transplant Therapeutics Consortium, which is managed by the Critical Path Institute.

OII's 2020 priorities included hiring for leadership and Medical Officer positions and establishing the new divisions of the newly formed office. OII staff were ultimately successful in working in a completely virtual environment, fulfilling their regular responsibilities in addition to the heavy COVID-19 workload.

## Office of Rare Diseases, Pediatrics, Urologic, and Reproductive Medicine (ORPURM)



ORPURM is a new OND clinical office comprised of four review divisions: DPMH, the Division of Rare Diseases and Medical Genetics (DRDMG), the Division of Urology, Obstetrics and Gynecology (DUOG), and DPT-RPURM/SM.

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One noteworthy new drug approval was DUOG's approval of a transdermal contraceptive patch evaluated in trials that enrolled a substantial proportion of reproductive age women who were overweight or obese, representative of U.S. women. This was the first time this has been done in contraceptive drug development history. The trials demonstrated an increased risk of venous thromboembolism and decreased efficacy in obese women, which resulted in labeling related to body mass index to ensure that the product's benefits outweigh its risks.

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DPMH oversees the study of drugs and therapeutic biologics in the pediatric, pregnant, and breastfeeding populations, and improves pregnancy, lactation-related, and pediatric information in product labeling. DPMH is actively engaged in research related to drug development in children, pregnant women, and lactating women, with research grants totaling over \$1.2 million in FY20 and spanning several years. In 2020, DPMH worked closely with OND review divisions and regulatory counterparts from the European Medicines Agency to work to include pregnant and pediatric patients of all ages in drugs and biologics being developed for COVID-19. DPMH also provided primary review support for pediatric issues related to the remdesivir development program and volunteered staff to serve on detail to help review COVID-19 applications in other OND divisions. DPMH developed numerous training opportunities to increase education around regulatory science in the development of drugs for children, pregnant and lactating women, including the three-part CDER Pediatric Reviewer Series planned in collaboration with OB/OTS. DPMH also partnered with the Children's National Medical Center to develop a 6-month training program in pediatric regulatory science for fellows and early career physicians.

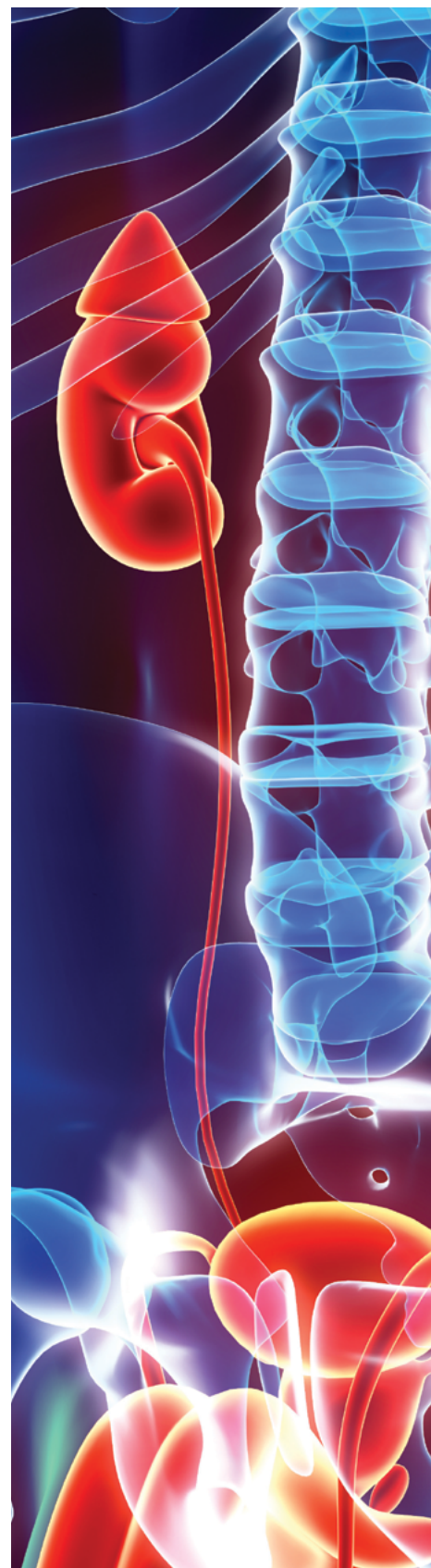
DRDMG, newly formed by combining expert clinical reviewers with a multi-disciplinary Rare Diseases Team, oversees drug development for patients with rare inborn errors of metabolism and serves as a hub for rare disease drug development across all of OND by coordinating

education, policy, research and stakeholder engagement. DRDMG's main priority in 2020 was facilitating adaptations and innovations in response to COVID-19 to ensure the continued success of drug development programs for patients with rare diseases, including decentralized trial designs and protocol modifications for virtual trial visits. DRDMG also approved the first treatment that extends the lives of patients with a rare, fatal, pediatric disease known as Progeria. DRDMG administrated the award of five Rare Disease Pediatrics Disease Priority Review Vouchers to incentivize rare pediatric disease drug product development.

DRDMG staff engaged with external stakeholders through attending and presenting at various virtual scientific meetings and patient listening sessions for rare diseases. DRDMG staff also hosted the 10th Annual Rare Diseases Reviewer Training in May 2020, with a record 417 participants and kicked off a new quarterly OND Rare Disease Seminar to foster innovation and knowledge across review divisions. Recent topics included lessons learned from pediatric and oncology rare disease clinical trial networks and the Rare Disease Cures Accelerator — Data and Analytics Platform. The team launched Zegram, a newsletter distributed across FDA highlighting innovative approaches, precedent-setting regulatory decisions, publications, and important upcoming events for rare disease drug development. The team also continued to lead the International Rare Diseases Cluster, which facilitates global regulatory alignment on rare disease trial design and added Health Canada as a new member.

DUOG assures safe and effective drugs and therapeutic biologics for urologic, obstetric, and gynecologic conditions. In 2020, DUOG co-authored several guidances, including one on [interstitial cystitis/bladder pain syndrome](#). This guidance is an important step supporting clinical drug development for this poorly understood chronic bladder pain condition with very limited treatment options. DUOG had several notable drug approvals, such as Oriahnn (a combination product consisting of elagolix, estradiol and norethindrone acetate), the first medical treatment for heavy menstrual bleeding associated with fibroids in women; and VESicare LS (solifenacin succinate), the first treatment for a form of bladder dysfunction approved in pediatric patients as young as two years old.


The OND reorganization created ORPURM as a new office, weaving in old and new divisions. Despite forming a new office amidst the COVID-19 pandemic, ORPURM transitioned seamlessly into a remote work environment and has been successfully building collaborations across its divisions to complete its review work and contribute to OND's COVID-19 response.



## OND Future Outlook

2020 has been a year of immense challenges and accomplishments for OND. We completed a substantial reorganization, with many new divisions and offices and new leaders, in March, just as the COVID-19 infection rates markedly increased. With rising infection rates in the community, our organization along with many other organizations and businesses moved to working virtually. As this Annual Report shows, despite a new organization and the huge workload from supporting and regulating development for therapeutics for COVID-19, OND was able to step up, continuing to meet and exceed many PDUFA review goals. Our staff knows that the work we do every day to bring safe and effective drugs to the American public, and the urgent need to find therapies to address the pandemic, were both of critical importance. The work OND does is, at its core, collaborative: collaboration across OND divisions and offices, and across CDER offices is an essential feature of drug review. On a day-to-day basis, OND staff participate in meetings reflecting their participation in numerous teams and working groups to evaluate INDs or NDAs/BLAs or post-approval safety issues, as well as to set policy or discuss research to advance drug regulatory sciences. Accomplishing these critically important objectives while working virtually is no easy task — but one that OND staff stepped up and achieved with “flying colors.” The breadth of what’s been accomplished in 2020 — from the approval of innovative new treatments meeting unmet needs, to key regulatory actions on approved drugs to assure their safe use, to a range of new guidances providing industry key information on development programs, to issuing EUAs for drugs to fight COVID-19, to numerous other efforts — and having achieved this while working virtually — is a reflection of the expertise, knowledge, analytic skills, energy, and dedication of OND staff. I can say, without hesitation, that what’s been achieved in 2020 tells us that the future outlook of OND is indeed promising.

It’s worth considering some of the challenges OND will face in 2021 and beyond. The landscape for drug development is changing. We can expect that the challenges of COVID-19 we faced in 2020 will continue in 2021, but, as the end of the pandemic is in sight, considering new challenges is important. We have already seen greater focus on development for rare diseases or disease subtypes shifting away from larger programs targeting common, chronic diseases — such a shift raises the need for new approaches to development, the need for new development tools, and new policy challenges. New platforms for drug development, such as siRNA, will also pose the need for new tools, guidances, and knowledge. The rise in interest in real world data and evidence to support regulatory decision-making means OND, along with many other CDER offices,



must gain additional experience to understand where and how such information can be appropriately used. The role of patients in drug development is also, appropriately, increasing. OND has always looked to patient input to inform regulatory decision-making; after all, our work is focused on making patient's lives better. So, understanding their perspectives and the ways in which their disease impacts their lives, is essential to our work and decisions. Over time, we must, therefore, continue to increase our efforts to engage with patients, learn from them, and utilize their input in our work.

CDER and OND have been carefully evaluating how to make our regulatory review processes more efficient and, ultimately, more effective given the many challenges we face. Over the past several years, the New Drug Regulatory Program (NDRP) has launched a number of workstreams and these are already yielding many changes in the organization of OND and the ways in which we work. The NDRP work on the integrated assessment, for example, aims for a more consistent, efficient team-based, and issue-focused assessment process and output, captured in a single FDA voice. The long-term outcomes for this objective include consistent development of highly integrated, thoroughly documented, and clearly communicated regulatory decisions. The new approaches emerging from the NDRP must be thoughtfully — and carefully — implemented into our day-to-day work and tested to be sure that they enhance our abilities to deliver on our objectives.

2020 has shown the resilience and the broad and deep capabilities of OND as an organization. As we face the ongoing demands of COVID-19, and the new demands of the changing drug development landscape, our organization is well poised to meet these, and other new, challenges.

## Appendix

### Office of New Drug Policy

#### Guidances

- [COVID-19: Developing Drugs and Biological Products for Treatment or Prevention](#); Final; May 2020.
- [COVID-19 Public Health Emergency: General Considerations for Pre-IND Meeting Requests for COVID-19 Related Drugs and Biological Products](#); Final; May 2020.
- [Geriatric Information in Human Prescription Drug and Biological Product Labeling](#); Draft; September 2020.

### Office of Drug Evaluation Sciences

#### Guidances

- [Patient-Focused Drug Development: Collecting Comprehensive and Representative Input](#); Final; June 2020.
- [Assessing COVID-19-Related Symptoms in Outpatient Adult and Adolescent Subjects in Clinical Trials of Drugs and Biological Products for COVID-19 Prevention or Treatment](#); Final; September 2020.
- [Eosinophilic Esophagitis: Developing Drugs for Treatment Guidance for Industry](#); Final; September 2020.
- [Conduct of Clinical Trials of Medical Products During the COVID-19 Public Health Emergency](#); Final; December 2020.

#### Presentations

- Presentations on Safety Analytics as Session Chair and Panel at the Drug Information Association meeting; June 2020.
- OND ORISE Fellow Summer Student Presentations (11 presentations); August 2020.
- Keynote Address at the PhUSE meeting on Safety Analytics; September 2020.
- OND Extramural Research Science Day (four talks); September 2020.
- Presentation and panel discussion on Core Outcome Sets at the Measuring What Matters Symposium of the International Society for Quality of Life Research; September 2020.
- OND ORISE Fellow Scientific Poster Day (38 posters); October 2020.
- [OND Research Webinar: Seeking Collaborators, Funding Opportunities Available](#); November 2020.

### Office of Therapeutic Biologics and Biosimilars

#### Guidances

- [Biosimilars and Interchangeable Biosimilars: Licensure for Fewer Than All Conditions of Use for Which the Reference Product Has Been Licensed](#); Draft; February 2020.



- [Promotional Labeling and Advertising Considerations for Prescription Biological Reference and Biosimilar Products Questions and Answers Guidance for Industry](#); Draft; February 2020.
- [Biosimilarity and Interchangeability: Additional Draft Q&As on Biosimilar Development and the BPCI Act](#); Draft; November 2020.

### Public Workshop/Meetings

- [Public Workshop: FDA/FTC Workshop on a Competitive Marketplace for Biosimilars](#); March 2020.
- [Public Meeting on the Reauthorization of the Biosimilar User Fee Act \(BsUFA\)](#); November 2020.

## Pharmacology/Toxicology

### Guidances

- [Nonclinical Safety Evaluation of the Immunotoxic Potential of Drugs and Biologics Guidance for Industry](#); Draft; February 2020.
- ICH S5(R3) Detection of Reproductive and Developmental Toxicity for Human Pharmaceuticals; Final; February 2020.
- ICH S11 Nonclinical Safety Testing in Support of Development of Pediatric Pharmaceuticals; Final; April 2020.
- [Nonclinical Testing of Orally Inhaled Nicotine-Containing Drug Products Guidance for Industry](#); Final; October 2020.

### Publications

- [Pivotal Considerations for Optimal Deployment of Healthy Volunteers in Oncology Drug Development](#); *Clinical Translational Science*; January 2020.
- [An FDA analysis of clinical hold deficiencies affecting investigational new drug applications for oncology products](#); *Regulatory Toxicology and Pharmacology*; February 2020.
- [Opportunities for use of one species for longer-term toxicology testing during drug development: A cross-industry evaluation](#); *Regulatory Toxicology and Pharmacology*; February 2020.
- [An FDA/CDER perspective on nonclinical testing strategies: Classical toxicology approaches and new approach methodologies \(NAMs\)](#); *Regulatory Toxicology and Pharmacology*; April 2020.
- [Performance of high-throughput CometChip assay using primary human hepatocytes: a comparison of DNA damage responses with in vitro human hepatoma cell lines](#); *Archives of Toxicology*; April 2020.
- [Toxicology Paradise: Sorting Out Adverse and Non-adverse Findings in Animal Toxicity Studies](#); *International Journal of Toxicology*; July 2020.
- [CD59-deficient bone marrow erythroid cells from rats treated with procarbazine and propyl-nitrosourea have mutations in the Pig-a gene](#); *Environ Mol Mutagen*; July 2020.
- [Drug Development 101: A Primer](#); *International Journal of Toxicology*; August 2020.

- [Performance of HepaRG and HepG2 cells in the high-throughput micronucleus assay for in vitro genotoxicity assessment](#); *J Toxicol Environ Health*; September 2020.
- [Summary of a workshop on preclinical and translational safety assessment of CD3 bispecifics](#); *Journal Immunotoxicology*; December 2020.

## Office of Infectious Diseases

### Guidances

- [COVID-19: Developing Drugs and Biological Products for Treatment or Prevention](#); Final; May 2020.
- [Cytomegalovirus in Transplantation: Developing Drugs to Treat or Prevent Disease](#); Final; May 2020.
- [Development of Anti-Infective Drugs for the Pediatric Population](#); Draft; June 2020.
- [Hospital-Acquired Bacterial Pneumonia and Ventilator-Associated Bacterial Pneumonia: Developing Drugs for Treatment](#); Final; June 2020.
- [Community-Acquired Bacterial Pneumonia: Developing Drugs for Treatment](#); Final; June 2020.
- [Limited Population Pathway for Antibacterial and Antifungal Drugs](#); Final; August 2020.

### Publications

- [Considerations and challenges in developing novel long-acting antiretrovirals modalities for treatment and prevention of HIV-1 infection: a regulatory perspective](#); *National Library of Medicine*; January 2020.
- [Hepatitis C Virus RNA Levels Following Virologic Failure With Direct-acting Antivirals: Implications for Lower Sensitivity Diagnostic Assays](#); *National Library of Medicine*; January 2020.
- [The varying specificity of urine cultures in different populations](#); *Cambridge University Press*; February 2020.
- [Geographic shifts in antibacterial drug clinical trial enrollment: implications for generalizability](#); *National Library of Medicine*; March 2020.
- [The Development of Therapeutics for the Treatment and Prevention of CMV Disease in the Transplant Population: A Regulatory Perspective](#); *National Library of Medicine*; March 2020.
- [Assessing Animal Models of Bacterial Pneumonia Used in Investigational New Drug Applications for the Treatment of Bacterial Pneumonia](#); *National Library of Medicine*; April 2020.
- [Association Between Seroclearance of Hepatitis B Surface Antigen and Long-term Clinical Outcomes of Patients With Chronic HBV Infection: Systematic Review and Meta-analysis](#); *National Library of Medicine*; May 2020.
- [Consensus on Language for Advance Informed Consent in Health Care-Associated Pneumonia Clinical Trials Using a Delphi Process](#); *National Library of Medicine*; May 2020.
- [Concordance of early and late endpoints for community-acquired bacterial pneumonia trials](#); *National Library of Medicine*; June 2020.
- [Achieving Regulatory Alignment for Anti-Infective Clinical Trials](#); *National Library of Medicine*; June 2020.

- [An Analysis of Antibacterial Drug Development Trends in the US, 1980 – 2019](#); *National Library of Medicine*; June 2020.
- [A SARS-like cluster of circulating bat coronaviruses shows potential for human emergence \(correction\)](#); *National Library of Medicine*; July 2020.
- [No Association Between DAA Treatment for HCV Infection and Herpes Zoster Infection in Analysis of Data From 37 Clinical Trials](#); *National Library of Medicine*; August 2020.
- [Treatment-Emergent Influenza Virus Polymerase Acidic Substitutions Independent of Those at I38 Associated With Reduced Baloxavir Susceptibility and Virus Rebound in Trials of Baloxavir Marboxil](#); *National Library of Medicine*; August 2020.
- [Optimizing pharmacology studies in pregnant and lactating women using lessons from HIV: a consensus statement](#); *American Society for Clinical Pharmacology and Therapeutics*; September 2020.
- [Trends in hospital-acquired and ventilator-associated bacterial pneumonia trials](#); *Clinical Infectious Diseases*; November 2020.
- [Advancing Animal Models for Antibacterial Drug Development](#); *Antimicrobial Agents and Chemotherapy*; December 2020.

## Workshops

- [Advancing Animal Models for Antibacterial Drug Development](#); March 2020.
- [Development Considerations of Antifungal Drugs to Address Unmet Medical Need](#); August 2020.
- [Coccidioidomycosis \(Valley Fever\): Considerations for Development of Antifungal Drugs](#); August 2020.
- Progressive Multifocal Leukoencephalopathy (PML) Clinical Trials Considerations Mini Symposium; September 2020.
- [Addressing Challenges in Inhaled Antifungal Drug Development](#); September 2020.

## Office of Oncologic Diseases

### Guidances

- [Hematologic Malignancies: Regulatory Considerations for Use of Minimal Residual Disease in Development of Drug and Biological Products for Treatment](#); Final; January 2020.
- [Inclusion of Older Adults in Cancer Clinical Trials](#); Draft; March 2020.
- [Cancer Clinical Trial Eligibility Criteria: Patients with Organ Dysfunction or Prior or Concurrent Malignancies](#); Final; July 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.

- [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.

## Publications

- [Comparison of iRECIST versus RECIST V.1.1 in patients treated with an anti-PD-1 or PD-L1 antibody: pooled FDA analysis](#); *J Immunother Cancer*; February 2020.
- [Differentiation Syndrome with Ivosidenib and Enasidenib Treatment in Patients with Relapsed or Refractory IDH-Mutated AML: An FDA Systematic Analysis](#); *Clin Cancer Res*; May 2020.
- [U.S. Food and Drug Administration: Initial Experience with the Real-Time Oncology Review Program](#); *Clin Cancer Res*; June 2020.
- [An FDA Analysis of Survival Outcomes Comparing an Adjuvant Paclitaxel and Trastuzumab Trial to an External Control from Historical Clinical Trials](#); *Ann Oncol*; August 2020.
- [Older adults in hematologic malignancy trials: Representation, barriers to participation and strategies for addressing underrepresentation](#); *Blood Rev*; September 2020.
- [CDK4/6 inhibitor treatment for patients with hormone receptor-positive, HER2-negative, advanced or metastatic breast cancer: a US Food and Drug Administration pooled analysis](#); *The Lancet Oncology*; December 2020.

## Workshops

- [FDA-AACR Workshop to Examine Under-Representation of African Americans in Multiple Myeloma Clinical Trials](#); February 2020.
- [FDA/CDER-AACR-IASLC Workshop to Address the Criticality of Tobacco Use Assessment in Oncology Therapeutic Trials](#); February 2020.
- [FDA/ASCO Clinical Outcomes Assessment in Cancer Clinical Trials](#); July 2020.

## Office of Neuroscience

### Publications

- [Assessment of similarity in antipsychotic exposure-response relationships in clinical trials between adults and adolescents with acute exacerbation of schizophrenia](#); *National Library of Medicine*; January 2020.
- [Developing an animal model to detect drug-drug interactions impacting drug-induced respiratory depression](#); *National Library of Medicine*; January 2020.
- [The trend of increasing placebo response and decreasing treatment effect in schizophrenia trials continues: An update from the US Food and Drug Administration](#); *National Library of Medicine*; March 2020.
- [Association of end point definition and randomized clinical trial duration in clinical trials of schizophrenia medications](#); *National Library of Medicine*; October 2020.
- [Association of Genetic Mutations and Loss of Ambulation in Childhood-Onset Dystrophinopathy](#); *National Library of Medicine*; November 2020.

## Presentations

- [International Society for CNS Clinical Trials and Methodology \(ISCTM\)](#); September 2020.
- Progressive Multifocal Leukoencephalopathy (PML) Clinical Trials Consideration Mini Symposium; September 2020.
- [American Academy of Child and Adolescent Psychiatry](#); October 2020.
- “To Hold or Not to Hold? Regulator and Sponsor Perspectives on Difficult FIH Toxicology Packages (Continuing Education Course); American College of Toxicology Annual Meeting (Virtual); November 2020.
- [“Nonclinical Safety Concerns for CBD in Commercial Products and Regulatory Recommendations for Cannabis-based Drug Product Development in the United States”](#); American College of Toxicology Annual Meeting (Virtual); November 2020.
- “FDA Role in the Regulation of Cannabis Products (and Insights Into IND Submission Challenges);” The Lambert Center for Medicinal Cannabis and Hemp, Thomas Jefferson University and Jefferson Health; (Virtual); November 2020.
- “A CDER Pharmacology Toxicology Perspective on the Safety Evaluation of Flavor Excipients;” FEMA Regulatory Affairs Virtual Committee Meeting; November 2020.
- [American College of Neuropsychopharmacology \(ACNP\)](#); December 2020.

## Office of Nonprescription Drugs

### Guidances

- [Restricted Delivery Systems for Oral Liquid Drug Products](#); Draft; March 2020.
- [Temporary Policy for Preparation of Certain Alcohol-Based Hand Sanitizer Products During the Public Health Emergency \(COVID-19\)](#); Final (Temporary); August 2020.
- [Temporary Policy for Manufacture of Alcohol-Based Hand Sanitizer Products During the Public Health Emergency \(COVID-19\)](#); Final (Temporary); August 2020.
- [Policy for Temporary Compounding of Certain Alcohol-Based Hand Sanitizer Products During the Public Health Emergency](#); Final (Temporary); August 2020.
- [Nonclinical Testing of Orally Inhaled Nicotine-Containing Drug Products](#); Final; October 2020.

### Publications

- [Effect of Sunscreen Application on Plasma Concentration of Sunscreen Active Ingredients: A Randomized Clinical Trial](#); *JAMA*; January 2020.
- [Suboptimal UVA attenuation by broad spectrum sunscreens under outdoor solar conditions contributes to lifetime UVA burden](#); *Photodermatology, Photoimmunology & Photomedicine*; January 2020.
- [In Vitro Testing of Sunscreens for Dermal Absorption: A Platform for Product Selection for Maximal Use Trials](#); *Journal of Investigative Dermatology*; April 2020.

- [FDA Initiative for Drug Facts Label for Over-the-Counter Naloxone](#); *New England Journal of Medicine*; May 2020.
- [Skin Cancer Prevention and Sunscreen Safety: Commentary on American Society of Clinical Oncology Policy Statement](#); *ASCO Journal*; August 2020.
- Alcohol-based hand sanitizer exposures and the effects on young children in the U.S. during the COVID-19 pandemic; *Clinical Toxicology*; August 2020.

### Presentations

- OTC Monograph Reform Presentations
  - Hosted a webinar: [Monograph Reform is Here! Learn What to Expect and How to Prepare](#); May 2020.
  - Presented at Consumer Healthcare Products Association (CHPA) meeting; September 2020.
  - Presented at Regulatory Affairs Professional Society (RAPS) Convergence 2020; September 2020.
  - Presented at Independent Beauty Association; September 2020.
  - Presented at American Academy of Pediatrics — Committee on Drugs; September 2020.
  - Presented at Food and Drug Law Institute; October 2020.
  - Monograph Reform Road Show; presented to multiple OND divisions.
- Enabling Consumer Decision Making in the Self-Care Environment; CHPA; September 2020.
- [Rx to OTC Switch — Expanding Self-Care by Enabling Consumer Decision-Making](#); RAPS Convergence; September 2020.

## Office of Specialty Medicine

### Publications

- [Nephrogenic Systemic Fibrosis Risk Assessment and Skin Biopsy Quantification in Patients with Renal Disease following Gadobenate Contrast Administration](#); *American Journal of Neuroradiology*; March 2020.
- [Creatinine-based renal function assessment in pediatric drug development: an analysis using clinical data for renally eliminated drugs](#); *Clinical Pharmacology and Therapeutics*; July 2020.
- Brain Tau Imaging — FDA Approval of Flortaucipir F18 Injection; *J of Nucl Med*; August 2020.
- [Cutaneous Radiation Injuries: Models, Assessment and Treatments](#); *Radiation Research*; August 2020.

### Presentations

- Navigating the Regulatory Gauntlet; Glaucoma 360 Annual Meeting; January 2020.
- Panel discussion: Developing MCMs to Treat Acute and Chronic Effects of Ocular Chemical Toxicity NIH Workshop; February 2020.
- Inflammation and Retinal Therapeutics; Stanford Retina Innovation Summit; August 2020.

- Biomarkers According to the Animal Rule; NIAID Biomarkers in Radiation Countermeasures and Biodosimetry Workshop; September 2020.
- Gadolinium Based Contrast Agents: Safety Considerations; Global Summit on Regulatory Science; September 2020.
- Renal Maturation and the Impact on Drug Therapies; American College of Clinical Pharmacology Annual Conference; September 2020.
- Ophthalmic Drug Regulation; University of Tennessee; October 2020.
- What FDA Expects in an Ocular GVHD Clinical Trial; Chronic GVHD Conference; October 2019.

### Public Meetings

- [FDA-SNMMI-MITA-WMIS Workshop — PET Inspection Management and Regulatory Issues](#); February 2020.
- [FDA-NRC workshop Enhancing Development of Emerging Technologies: Radiopharmaceuticals and Radiological Devices](#); October 2020.

## Office of Cardiology, Hematology, Endocrinology, and Nephrology

### Guidances

- [Type 2 Diabetes Mellitus: Evaluating the Safety of New Drugs for Improving Glycemic Control](#); Draft; March 2020.
- [International Conference on Harmonization E14 and S7B Clinical and Nonclinical Evaluation of QT/QTc Interval Prolongation and Proarrhythmic Potential](#); Draft; September 2020.

### Publications

- [Clinical and regulatory landscape for cardiogenic shock: A report from the Cardiac Safety Research Consortium ThinkTank on cardiogenic shock](#); *Am Heart J*; January 2020.
- [Transcranial Doppler Screening Adherence among Children with Sickle Cell Anemia Seen in the Emergency Department](#); *J Pediatr*; February 2020.
- [Effects of Electrical Stimulation on hiPSC-CM Responses to Classic Ion Channel Blockers](#); *Toxicol Sci*; April 2020.
- [Characteristics and outcomes of osteomyelitis in children with sickle cell disease: A 10-year single-center experience](#); *Pediatr Blood Cancer*; May 2020.
- [Endpoints in Heart Failure Drug Development: History and Future](#); *JACC Heart Fail*; June 2020.
- [Ask the Expert: A Regulatory Perspective on Clinical Trials for Pulmonary Arterial Hypertension](#); *Advances in Pulmonary Hypertension*; August 2020.
- [FDA approval summary: Dalteparin for the treatment of symptomatic venous thromboembolism in pediatric patients](#); *Pediatr Blood Cancer*; September 2020.
- [Cancer Risk Associated with Lorcaserin; The FDA's Review of the CAMELLIA-TIMI 61 Trial](#); *Engl J Med*; September 2020.

- [Methods for Employing Information About Uncertainty of Ascertainment of Events in Clinical Trials](#); *Ther Innov Regul Sci*; September 2020.
- [Assessing the Safety of Glucose-Lowering Drugs; A New Focus for the FDA](#); *N Engl J Med*; September 2020.
- FDA Approval Summary: Ruxolitinib for Treatment of Steroid-Refractory Acute Graft-Versus-Host Disease; *Oncologist*; October 2020.
- [General Principles for the Validation of Proarrhythmia Risk Prediction Models: An Extension of the CiPA In Silico Strategy](#); *Clin Pharmacol Ther*; November 2020.

### Public Meetings/Presentations

- Multiple webinars and live meetings for the Heart Failure Collaboratory, the Heart Valve Collaboratory, Cardiovascular Clinic Trialists Forum, Kidney Disease Clinical Trialists, Pulmonary Hypertension, and others (DCN).
  - Heart Failure Collaboratory; March, April, September, October 2020.
  - Pulmonary Vascular Research Institute (pulmonary hypertension); June 2020.
  - Heart Valve Collaboratory; August 2020.
  - Kidney Disease Clinical Trialists; September 2020.
  - Cardiovascular Clinical Trialists; December 2020.
- DNH partnered with Office of Minority Health and participated in the Baltimore Health Expo to highlight the importance of maintenance of good health for patients with sickle cell disease; March 2020.
- Mini-Symposium on the role of C-peptide in clinical trials for type 1 diabetes (DDLO); April 2020.
- DNH staff is partnering with Centers for Disease Control Blood Disorders Program to facilitate drug development for sickle cell disease; ongoing meetings with CDC.

### Office of Immunology and Inflammation

#### Guidances

- Eosinophilic Esophagitis: Developing Drugs for Treatment — Guidance for Industry; Final; September 2020.

#### Presentations

- [Overview of Patient-Focused Drug Development](#); National Pancreas Foundation Externally-Led Patient-Focused Drug Development Meeting on Pancreatitis; March 2020.
- [Demographic And Clinical Characteristics Of Asthma Patients With Anti-interleukin-5 Therapy In The Sentinel Distributed Database](#); 2020 American Thoracic Society meeting; May 2020.
- [Demographic And Clinical Characteristics Of COPD Medication Users In The Sentinel Distributed Database](#); 2020 American Thoracic Society meeting; May 2020.



- [Incidence Of Ocular Adverse Events Associated With Mometasone Implants Among Patients With Nasal Polyposis](#); ISPE's 36th International Conference on Pharmacoepidemiology and Therapeutic Risk Management (ICPE); September 2020.
- [PFDD on Systemic Sclerosis](#); October 2020.
- [American College of Rheumatology Convergence](#); American College of Rheumatology; November 2020.
- FDA Safety Session: Updates from the FDA; November 2020.
- [Pediatric Rheumatic Diseases Drug Development: Challenges & Opportunities](#); November 2020.
- [Clinical Research Conference: Optimizing RCTs in Rheumatology: Past, Present and Future: Surrogate Outcomes & Indirect Comparisons: FDA Perspective](#); November 2020.

## Office of Rare Diseases, Pediatrics, Urologic, and Reproductive Medicine

### Guidances

- [Mucopolysaccharidosis Type III \(Sanfilippo Syndrome\)](#); Draft; February 2020.
- [Slowly Progressive, Low-Prevalence Rare Diseases with Substrate Deposition that Results from Single Enzyme Defects](#); Final; March 2020.
- [Development of Anti-Infective Drug Products for the Pediatric Population](#); Draft; June 2020.
- [Pregnancy, Lactation, and Reproductive Potential: Labeling for Human Prescription Drug and Biological Products – Content and Format Guidance for Industry](#); Draft; July 2020.
- [Pediatric Study Plans: Content of and Process for Submitting Initial Pediatric Study Plans and Amended Initial Pediatric Study Plans Guidance for Industry](#); Final; July 2020.
- [Pregnancy, Lactation, and Reproductive Potential: Labeling for Human Prescription Drug and Biological Products – Content and Format, Contributor](#); Draft; July 2020.
- [Premenopausal Women with Breast Cancer: Developing Drugs for Treatment, Contributor](#); Draft; October 2020.

### Publications

- [Extrapolation of Adult Efficacy to Pediatric Patients with Chemotherapy-Induced Nausea and Vomiting](#); *J Clin Pharmacol*; January 2020.
- [Scientific and Regulatory Considerations for an Ontogeny Knowledge Base for Pediatric Clinical Pharmacology](#); *Clin Pharmacol Ther*; January 2020.
- [Clinicians' Perspective of the New Pregnancy and Lactation Labeling Rule \(PLLR\): Results from an AAAAI/FDA Survey](#); *The Journal of Allergy and Clinical Immunology: In Practice*; February 2020.
- [Defining the path ahead for NOAC use in the pediatric population: A cardiac safety research consortium think tank](#); *American Heart Journal*; February 2020.

- [Exposure-Response Assessment in Pediatric Drug Development Studies Submitted to the US FDA](#); *Clin Pharmacol Ther*; March 2020.
- [Endpoints for Clinical Trials in Primary Hyperoxaluria](#); *Clinical Journal of the American Society of Nephrology* ePress.; March 2020.
- [Pediatric Extrapolation in Type 2 Diabetes: Future Implications of a Workshop](#); *Clinical Pharmacology & Therapeutics*; March 2020.
- [Combined Pediatric and Adult Trials Submitted to the US Food and Drug Administration 2012–2018](#); *Clin Pharmacol Ther*; May 2020.
- [Dosing Recommendations for Pediatric Patients with Renal Impairment](#); *The Journal of Clinical Pharmacology*; June 2020.
- [Approaches to Dose Finding in Neonates, Illustrating the Variability between Neonatal Drug Development Programs](#); *Pharmaceutics*; July 2020.
- [Application of physiologically based pharmacokinetic modeling for sertraline dosing recommendations in pregnancy](#); *Npj Systems Biology and Applications*; November 2020.
- [Withdrawing Approval of Makena – A Proposal from the FDA Center for Drug Evaluation and Research](#); *The New England Journal of Medicine*; December 2020.
- [The Importance of Clinical Research in Pregnant Women to Inform Prescription Drug Labeling](#); *Clin Pharmacol*; December 2020.

## Presentations

- [Your Voice Matters: How to Engage with the FDA](#); NORD Webinar; January 2020.
- International Regulators' meeting on Pregnancy and Lactation; MHRA; January 2020.
- Netherlands Organization for Applied Scientific Research Session on Micro-dosing in Pregnancy; January 2020.
- [Patient Listening Session, Hunter Syndrome](#); February 2020.
- [Makena – FDA's Review and Assessment](#); 40th Annual Society of Maternal Fetal Medicine meeting; February 2020.
- Drug Development in Rare Diseases at FDA; BIOCON; March 2020.
- Task Force on Research Specific to Pregnant Women and Lactating Women (PRGLAC); March 2020.
- NIH Rare Disease Clinical Research Network Steering Committee; April 2020.
- [Pediatric COVID-19 Webinar hosted by the Institute for Advanced Clinical Trials \(I-ACT\) for Children](#); May 2020.
  - Approach to Pediatric Therapeutics Development in Pediatric COVID-19; May 2020.
  - Innovative Methods for Conducting Pediatric Clinical Trials; May 2020.

- [ICH and Pediatric Therapeutics Development: A Win for Children around the World, DIA annual meeting \(Virtual\)](#); June 2020.
- [Patient Listening Session – Homocystinuria \(HCU\)](#); June 2020.
- [Pediatric Development During COVID-19: Can Increasing Collaboration Lead to Fewer Unnecessary Clinical Trials? FDA Perspectives, DIA annual meeting \(Virtual\)](#); June 2020.
- Organization of Teratology Information Specialists-Society for Birth Defects Research and Prevention annual meeting: presentation on PRGLAC and FDA efforts to advance research in Pregnant and Lactating Women; July 2020.
- Corporate Council, National Organization for Rare Diseases; July 2020.
- [Pompe Disease Patient-Focused Drug Development Meeting \(Virtual\)](#); July 2020.
- [Family Support Conference, National Niemann-Pick Disease Foundation](#); July 2020.
- Scientific Advisory Board, National Niemann-Pick Disease Foundation; July 2020.
- Duke-Margolis, Rare Disease Clinical Trial Data Sharing (Virtual); August 2020.
- “COVID-19 and FDA” to the Committee on Drugs; American Academy of Pediatrics; August 2020.
- NORD/FDA COVID-19 Impact on the Rare Disease Community; September 2020.
- [American Course on Drug Development and Regulatory Sciences: Pediatric Extrapolation: Little by Little Becomes a Lot](#); September 2020.
- Organizing committee and moderator of the COVID-19 in Pregnancy: Clinical, Research and Therapeutics Updates public virtual workshop, collaboration with NICHD; September 2020.
- [Rare Disease Cures Accelerator Workshop \(Virtual\)](#); October 2020.
- [NORD Rare Diseases & Orphan Products Breakthrough Summit](#); October 2020.
- [Informa Connect-CBI FDA/CMS Summit \(Virtual\)](#); December 2020.

**Office of New Drugs**  
Center for Drug Evaluation and Research  
U.S. Food and Drug Administration  
10903 New Hampshire Avenue  
Silver Spring, Maryland 20993  
[www.fda.gov](http://www.fda.gov)