

2018
ANNUAL
REPORT

PARTNERSHIP
in
— ACTION

REAGAN - UDALL
FOUNDATION
FOR THE FDA



REAGAN-UDALL
FOUNDATION
FOR THE FDA

WELCOME LETTER

We begin by saying thank you. Thank you to our partners and collaborators who helped make 2018 a highly productive and successful year for the Reagan-Udall Foundation for the FDA. And thank you to June Wasser, our former Executive Director. We appreciate the partnerships she built, the programs she nurtured, and the advancements she led.



2018 was a time of growth and evolution for the Foundation. Our flagship programs, Innovation in Medical Evidence Development and Surveillance (IMEDS) and the Expanded Access Navigator, matured and evolved, and we introduced new initiatives, such as our first topic-based public meeting at the U.S. Food and Drug Administration (FDA). Through it all, one thing was constant: the engagement of partners who are invested in our work and are willing to take action.

Such public-private partnerships are the hallmark of the Foundation, extending our combined impact beyond what any of us could achieve alone. These powerful collaborations help keep the FDA informed about rapidly changing science, ready to evaluate it, and move it safely into our everyday lives.

As you read this report, you will see the results of the Foundation's partnerships: publication of IMEDS studies adding to collective safety knowledge, inclusion of rare disease information in the Expanded Access Navigator to help often-overlooked patients, exploration with FDA of an exclusive curriculum for our Fellowship in Regulatory Science Training program, and dissemination of our public meeting report highlighting practical strategies for Leveraging Real-World Treatment Experience from Expanded Access Protocols.

This is what Partnership in Action looks like.

We began the letter with a thank you and conclude with a promise. In 2019, the Foundation will continue to foster stakeholder dialogue and build collaborative opportunities to support and inform the FDA's mission to advance innovation and modernize regulatory science.

Together, we play a vital role in the health, safety, and security of American lives.

Sincerely,

A handwritten signature in black ink, appearing to read "Ellen V. Sigal". The signature is fluid and cursive.

Ellen V. Sigal, PhD

Board Chair

ABOUT THE FOUNDATION

Created by a nonpartisan act of Congress, [the Reagan-Udall Foundation for the FDA](#) is an independent 501(c)(3) not-for-profit organization charged with advancing regulatory science to help the U.S. Food and Drug Administration accomplish its mission. The Foundation works to improve America's public health through public-private partnerships that facilitate innovation,

foster the use of real-world evidence, and identify modern tools and policies to keep pace with today's rapidly evolving science.

Putting Partnership in Action, the Foundation serves as a bridge bringing together stakeholders from FDA-regulated industries, academia, patient advocacy, physician groups, and government to build collaboration, provide insight, and take on modern public health challenges.

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Our unique relationship with the Reagan-Udall Foundation for the FDA allows us to work more broadly and leverage public-private partnerships where interests align.

RADM DENISE HINTON, Chief Scientist, U.S. Food and Drug Administration

MESSAGE FROM THE 23RD COMMISSIONER

Engagement and collaboration are at the core of the Reagan-Udall Foundation for the FDA, a key partner in advancing the U.S. Food and Drug Administration's (FDA) mission to modernize product development, accelerate innovation, and enhance product safety.



Public-private partnerships can help us tackle major public health problems and inform the work of FDA. The Foundation's Innovation in Medical Evidence Development and Surveillance (IMEDS) program illustrates how creating efficient, collaborative environments can extend the FDA's reach: industry scientists are now leveraging the Sentinel Network to address regulatory decision making.

Patients are also at the center of our joint commitment to expanded access to investigational therapies, which is why we clarified last year that drug companies posting their expanded access policies and contact information in the Foundation's Expanded Access Navigator Company Directory meet the federal requirement of making policies "public and readily available" to physicians and patients who fear they may have run out of options. The Foundation's November meeting, Leveraging Real-World Treatment Experience from Expanded Access Protocols, provided insight to FDA and industry thinking that will not only inform regulatory decisions but may also help the next patient — and others who follow — live longer lives.

We are experiencing an exceptional moment in history: science is progressing rapidly and new medical opportunities require visionary regulatory approaches. Our unique statutory relationship with the Foundation is one way FDA keeps pace with scientific advancement and ensures that regulatory science makes life meaningfully better.

Sincerely,

A handwritten signature in black ink that reads "Scott Gottlieb MD". The signature is fluid and cursive, with the first letters of the first and last names being capitalized and prominent.

Scott Gottlieb, MD

23rd Commissioner of Food and Drugs



Nearly 300 stakeholders pack the Great Room at FDA's White Oak Campus

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Everybody who has a role in expanded access was represented in that room. Patients, providers, regulators, industry — everybody. The Foundation was able to bring everyone together and that's a testament to the importance of the topic and the relevance of the Foundation.

PAUL MELMEYER, National Organization for Rare Disorders

LEVERAGING REAL-WORLD TREATMENT EXPERIENCE FROM EXPANDED ACCESS PROTOCOLS

Sharing stakeholder perspectives and taking deep dives into critical issues advance the Foundation's charge to inform and support the FDA mission. In 2018, we convened our first topic-based public meeting, drawing nearly 300 stakeholders from government, industry, academia, healthcare, and patient advocacy to the FDA's White Oak Campus to explore [Leveraging Real-World Treatment Experience from Expanded Access Protocols](#).

“The Reagan-Udall Foundation plays a pivotal role in expanded access — most importantly as a convener to drive important discussion,” says Paul Aliu, PharmD, MBA, Global Head of Medical Governance at Novartis. “No other group is placed to do just that. No one else has the reach and the credibility to engage regulators in open discussion with industry, patients, and physicians.”



➤ Commissioner Gottlieb discusses real-world evidence

Bookending the meeting were FDA officials who delivered a simple message: expanded access informs rather than hurts a product's regulatory profile. While the primary purpose of expanded access is to serve patients who are out of treatment options, the process also presents an opportunity to collect real-world data that informs payors, industry, regulators, physicians, and ultimately, other patients.

Three panels featuring 16 speakers representing varied stakeholder views discussed the challenges and bioethics of collecting expanded access data, but each overwhelmingly heralded the value such real-world data can provide — especially in reaching the shared goal of getting safe therapies to market faster so more patients benefit.

A series of strategies emerged from the meeting; among them, that expanded access data collection needs to be

standardized to leverage its utility, but formal regulation is not needed given the distinct nature of each expanded access program. Rather, regulators recommended focusing on only a few clinically meaningful data points that can be accurately and consistently collected. The full list of strategies is published in the [meeting report](#) along with a recommendation to establish best practices in expanded access data collection.

RADM Denise Hinton, FDA's Chief Scientist, called the meeting "invaluable," noting that "this kind of discussion helps us better understand challenges in the field, but also helps participants get a better understanding of FDA's willingness to work with them." Hinton called on the Foundation to host similar events on other key topics "because these discussions provide necessary insight and complement the priorities of the FDA."

How Results Could be Incorporated into the Regulatory and Product Review Activities of the FDA

The meeting report is being shared with FDA and broadly disseminated to stakeholders, including patient groups and the biopharmaceutical industry, with the intention of implementing data strategies and encouraging participation in pilot studies to identify best practices for expanded access data collection.



➤ Sharing patient, physician, and bioethics perspectives



➤ Industry panelists discuss how they use expanded access data



Expanded Access Convening

FDA regulators, with 20th FDA Commissioner Andrew C. von Eschenbach, stress that expanded access data can enhance a drug's regulatory profile rather than threaten it.



Stakeholders provide input on the expanded access process



Expanded access programs are crucial to people living with rare diseases who often don't have any other options. The Navigator makes it easier for patients and physicians to gain early access to potential life-saving therapies by providing comprehensive guidance and links to industry information all in one place.

JULIA JENKINS, Executive Director,
EveryLife Foundation for
Rare Diseases

EXPANDED ACCESS NAVIGATOR

Crafting meaningful public-private partnerships is core to the Foundation's mission, and from its origin, the [Expanded Access Navigator](#) is an example of that approach. Collaborating with FDA, patient advocates, physicians, and the biopharmaceutical industry allows the Foundation to evolve the Navigator's online resources to meet the changing and newly-identified needs of all those engaged in the expanded access ecosystem.

Background

Launched in 2017 in partnership with the FDA, the Expanded Access Navigator puts everything in one place: company contacts, a glossary, and step-by-step guides for patients and physicians. A unique feature of the Navigator is the [Company Directory](#), which helps users identify biopharmaceutical companies with expanded access programs and view their specific policies and criteria. "The Navigator, and especially the Directory, fills an important niche," notes RADM Denise Hinton, Chief Scientist at FDA.

In fall 2018, the FDA announced that listing a company's expanded access policy in the Navigator's Company Directory meets the 21st Century Cures Act requirement of making expanded access policies "readily available." Such listings ease access to information, help inform physician-patient decision making, and increase health equity by making information easy to find and easy to understand.

Goals

The Expanded Access Navigator serves as a roadmap to help patients and physicians move through the single-patient expanded access process and connect directly with companies providing investigational therapies. The goals of the Navigator are to:

- Increase understanding of expanded access by filling information gaps and clarifying misconceptions
- Reduce the burden on physicians and patients of finding expanded access information
- Increase willingness of physicians to explore expanded access for their patients
- Facilitate and expedite the request process
- Increase equity by ensuring that every patient is aware of expanded access as an option
- Improve physician and patient experience
- Assist FDA and industry in helping physicians better navigate the expanded access process

Achievements

At the initial launch of the Navigator, much of the information was oncology focused as cancer patients comprise a large percentage of expanded access requests. Yet patients with rare diseases also are in need of the Navigator's information; they are often among the first to run out of treatment options because of the uncommon nature of their conditions and limited drug development pipelines. In 2018, the Foundation closely collaborated with the rare disease community to expand the Navigator, recruiting biopharmaceutical companies working in the rare disease space to list in the Navigator's Company Directory and adding webinars, infographics, and other important resources. "Information in the Navigator is especially important for people dealing with rare, life-threatening diseases," says Amicus Therapeutics' Chief Patient Advocate, Jayne Gershkowitz. "These individuals and their caregivers are often overwhelmed and may not have a lot of choices or know the potential choices that exist. Expanded access information, presented clearly, in an easy to understand and well-organized manner, can truly make a difference — one that could be life-saving for some."

A postcard campaign with the National Organization for Rare Disorders and other rare disease advocates and demonstrating the Navigator at the National Institutes of Health's Rare Disease Day program helped raise the profile of the online program, leading to a 12 percent increase in use and unique page views. Company listings grew by eight percent.

The Foundation also convened a meeting of stakeholders from FDA and industry in July 2018 to explore enhancements to make the Navigator more useful in connecting patients and physicians to industry and streamlining the application process. As a result, the Foundation began scoping two projects: importing expanded access data from ClinicalTrials.gov so users do not have to bounce between websites for expanded access details,

and creating a [company guide](#) to help small or emerging companies with limited resources and experience create their own expanded access policies and procedures. Both enhancements are set to launch in early 2019.

Governance and Use of Funds

A small group of stakeholders invested \$45,000 to support the expansion of the Expanded Access Navigator to serve patients with rare diseases.

How Results Could be Incorporated into the Regulatory and Product Review Activities of the FDA

The Navigator can inform and complement Project Facilitate, a new pilot being planned by the Oncology Center for Excellence (OCE) at FDA to assist physicians in applying for expanded access and to track outcomes. The Foundation and OCE are working together to gather stakeholder input and establish linkages that will improve patient access to investigational therapies and may create a physician-support model across disease states.

The Division of Drug Information at CDER reports regularly referring people to the Navigator and the Navigator was featured in CDER's webinar, "An Overview of FDA's Expanded Access Program," as a tool for providers, patients, and industry. CDER reports a slight increase in the number of expanded access requests it processes and continues to allow more than 99 percent of expanded access requests to proceed.





INNOVATION IN MEDICAL EVIDENCE DEVELOPMENT AND SURVEILLANCE (IMEDS)

One goal of scientific collaboration is finding answers to previously unanswered questions. The [Innovation in Medical Evidence Development and Surveillance](#) (IMEDS) program, a transformational public-private partnership led by the Foundation, mobilizes data providers, drug manufacturers, researchers, and the FDA to accelerate research and answer critical patient safety questions.

“IMEDS makes good on FDA’s commitment to Congress and to the public to make tools and resources available to answer real-world safety questions affecting broad patient populations,” says Jacqueline Corrigan-Curay, MD, JD, Director of the Center for Drug Evaluation and Research’s Office of Medical Policy at FDA.

Background

FDA tasked the Foundation with making the Sentinel network into a highly utilized national resource for the broader regulatory community. Launched in January 2017, IMEDS allows industry and other researchers to conduct

post market analyses of drugs using one of the nation’s largest claims databases supplemented by FDA-validated methods and tools.

The Foundation facilitates collaboration among study sponsors, data partners, and the analytic center at Harvard Pilgrim Health Care to address important regulatory questions and requirements. “IMEDS studies are true collaborations: it’s more than data; you get scientific partners,” stresses Rachel E. Sobel, DrPH, MPH, Epidemiology Group Lead at Pfizer.

Goals

IMEDS research can help drug manufactures fulfill regulatory obligations; conduct population characterization studies, even for hard-to-reach populations like those with rare diseases; and engage in comparative effectiveness analyses.

“Clinical trials simply aren’t designed to answer the questions we can address through IMEDS,” says Syed Islam, DrPH, MSPH, MPH, Senior Medical





Because it relies on common and transparent procedures and infrastructure that can be understood by all participants, IMEDS appropriately shifts the focus from debates over differing methods and data to the underlying clinical and public health questions of concern.

SCOTT GOTTLIEB, MD

23rd Commissioner of Food and Drugs in Congressional testimony, July 2018

Director, Epidemiology Therapeutic Lead for Immunology and CV Medicine at AbbVie. “We can access millions of people, create cohort studies, and capture linkages to inform critical areas of public health—all while working with experts who know FDA-quality data.”

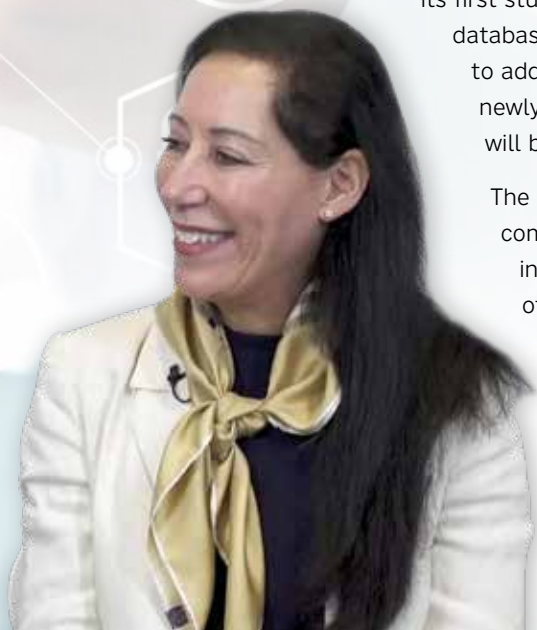
Achievements

In July 2018, FDA Commissioner Dr. Scott Gottlieb told the U.S. House of Representatives Energy and Commerce Committee’s Subcommittee on Health that “FDA is confident that IMEDS sponsors will play a key role in shaping the future of evidence generation to help answer outstanding questions about the safe and effective use of medical products in a broad range of populations.”

Dr. Gottlieb’s comments came as IMEDS extended its data reach even more, launching its first study to include the Centers for Medicare and Medicaid Services (CMS) database. Three new long-term studies were launched with industry partners to address EMA and FDA post market commitments, two of which involve newly approved drugs. As with all IMEDS studies, results of these projects will be released into the public domain after completion.

The Foundation regularly promotes the availability and value of IMEDS to companies and also works to identify collaborative project opportunities, including a Validation of Health Outcomes, Exposures, and Cohorts of Interest study to validate algorithms using the 10th revision of

➤ Former Executive Director June Wasser is interviewed by DIA Senior Scientist Raleigh Malik about how IMEDS helps answer drug safety and other pharmacovigilance research questions



TYPES OF DATA USED IN IMEDS



Office visit encounters



Emergency department visits



Inpatient hospital stays



Prescription drug utilization



Laboratory results

IMEDS

STEERING COMMITTEE

A diverse Steering Committee comprised of FDA, industry, academia, patient advocacy, and data partner representatives is charged with providing strategic guidance to IMEDS. The Committee advises on key sustainability and growth strategies in addition to exploring varied models of stakeholder collaboration.



IMEDS (CONTINUED)

International Classification of Diseases codes. IMEDS was showcased at the DIA Global Annual Meeting in a panel discussion with IMEDS collaborators and the Foundation's Executive Director, who was also featured in an [IMEDS interview](#) broadcast during the meeting. In August 2018, the Foundation highlighted program growth and previewed study opportunities for companies at a special luncheon hosted during the 34th Annual Conference on Pharmacoepidemiology and Therapeutic Risk Management in Prague.

Governance and Use of Funds

The Foundation reviews and facilitates all IMEDS studies, obtains Institutional Review Board approvals, provides scientific oversight, and manages business functions. Funds raised in 2018 totaled \$1,013,333 in financial support and funded research.

How Results Could be Incorporated into the Regulatory and Product Review Activities of the FDA

IMEDS study results are being used by companies for new drug submissions and to meet post market requirements and commitments. For example, Eli Lilly and Company included IMEDS study results estimating the incidence of venous thromboembolism in patients taking baricitinib in its resubmission to the FDA, and presented the findings at Sentinel's 2018 Public Meeting. Claudia Salinas, PhD, Senior Research Scientist at Eli Lilly and Company and the Principal Investigator on the study, has said she finds the replicability of IMEDS results reassuring for both industry and regulators.

Andrew Bate, PhD, Senior Director, Epidemiology Group Lead for Analytics at Pfizer, agrees, "With IMEDS, we're accessing regulatory-grade observational data that's best in class. That gives us confidence in a holistic system that FDA is comfortable with. Our study will be as good as it can be and will have strong alignment with the tools, methods, and data that FDA trusts."

**Pharmacoepidemiology
AND DRUG SAFETY**

MARCH 2018

Results of the IMEDS' pilot study published in [Do FDA label changes work? Assessment of the 2010 class label change for proton pump inhibitors using the Sentinel System's analytic tools](#)

— Pharmacoepidemiology and Drug Safety

MARCH 2018

IMEDS methods research supported by the Foundation, Sentinel, and the FDA featured in [Sequential surveillance for drug safety in a regulatory environment](#)

— Pharmacoepidemiology and Drug Safety

Annals of the
**Rheumatic
Diseases**

JUNE 2018

IMEDS study results published in [SAT0140 Risk of venous thromboembolism in rheumatoid arthritis patients treated with biologic and non-biologic dmards](#)

— Annals of the Rheumatic Diseases

CENTER FOR DRUG EVALUATION AND RESEARCH
**DRUG SAFETY
PRIORITIES
2018**

OCTOBER 2018

IMEDS highlighted by [FDA's Center for Drug Evaluation and Research](#)

— Drug Safety Priorities 2018 Report



The NEW ENGLAND
JOURNAL of MEDICINE

NOVEMBER 2018

IMEDS featured in [The FDA Sentinel Initiative — An Evolving National Resource](#)

— The New England Journal of Medicine



FiRST would prepare a 21st Century workforce to apply a deep understanding of regulatory science at FDA and related government agencies, industry, academia, and other research venues.

FELLOWSHIP IN REGULATORY SCIENCE TRAINING (FiRST)

Regulatory science is developing at a rapid pace creating a demand for experts who can transform the evaluation of tomorrow's cutting-edge innovations. Cultivating that next generation of regulatory specialists drives the strategy of the Reagan-Udall Foundation for the FDA's [Fellowship in Regulatory Science Training](#) (FiRST) program.

Goal

Designed to meet the broad needs of the 21st century workforce, FiRST is being explored jointly by the Foundation and FDA to foster a better understanding of regulatory oversight by providing future scientific leaders hands-on experience in regulatory science and policy operations.

Background

Through a competitive application and interview process, the Joint Steering Committee — comprised of Foundation and FDA experts — would select Fellows to be placed across FDA centers to assist FDA's work in modernizing regulatory science to foster medical and consumer product innovation. Each Fellow would work with an FDA mentor to craft a training and research plan to further their scientific development and build the practical skills needed to evaluate the safety and efficacy of products regulated by FDA.

After completing two years at FDA, Fellows would then have the option of spending the third year of their Fellowship at FDA, training at an industry partner, or pursuing a Master



of Public Health degree with an academic partner. Along the way, Fellows would have access to data, national experts, and top-level training to build unrivaled acumen and insight in regulatory processes, opportunities, and hurdles. FiRST would inform their perspectives and work throughout their careers.

Achievements

After gaining feedback from FDA officials, academics, industry, and potential Fellow candidates early in 2018, the Foundation refined the scope of the FiRST program plan and drafted the application and guidelines for Fellows. Foundation leadership also initiated fundraising efforts.

Governance and Use of Funds

While collaborating with FDA on curriculum and candidate selection, the Foundation leads the development and management of FiRST. The program is in the planning

stage with the Foundation in early conversations with potential sponsors.

How Results Could be Incorporated into the Regulatory and Product Review Activities of the FDA

FiRST Fellows would be immersed in regulatory science, so they could foster a better understanding of FDA's work in their post-fellowship employment, whether joining the FDA workforce or informing regulatory preparation and response activities in industry. FiRST graduates also could tackle challenging research in academic or nonprofit settings, which might be used by both regulators and industry to improve product development and ensure safety.



HANDS ON: FOOD FUNDAMENTALS

Our messages are often strongest when we lend expertise to others. The Reagan-Udall Foundation for the FDA served as an advisor on an innovative project teaching middle school students to think critically about the origin and safety of the foods they eat.

The Foundation's Executive Director advised on the Hands On: Real World Lessons for Middle School Classrooms — Food Fundamentals curriculum produced by the Grocery Manufacturers Association Science and Education Foundation (GMA SEF). The curriculum introduces students to how food

moves from farm to fork, including the role of safety regulation.

“The Reagan-Udall Foundation was the obvious partner when we needed a public health perspective for our Hands On: Food Fundamentals curriculum,” according to Weiyi Zhao, Director of Foundation Operations at GMA SEF. “Our collaboration helps educate future consumers at a critical time in their development.” GMA SEF reports awareness of food, particularly among youth, is low, and food related education in the



formative middle school years is a proven catalyst to prevent foodborne illness.

The Food Fundamentals module, expected to be in classrooms for the 2019–2020 school year, is part of a broader curriculum series currently used in 42 states reaching more than 76,000 students. Tied to National Science Education Standards, the curriculum is offered free of charge through GMA SEF.



Attendees at the 2018 Annual Public Meeting focus on FDA priorities

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The Foundation’s role in “creating a venue where interaction can occur about the underlying science is critical.”

ROBERT CALIFF, MD, MACC, 22nd Commissioner of Food and Drugs

EVOLUTION OF FDA SCIENCE AND ENGAGEMENT: THE REAGAN-UDALL FOUNDATION FOR THE FDA’S 2018 ANNUAL PUBLIC MEETING

The 2018 annual [public meeting](#) brought stakeholders together for a wide-ranging conversation with four FDA Commissioners: then-current Commissioner Dr. Scott Gottlieb and former Commissioners Dr. Robert Califf, Dr. Andrew C. von Eschenbach, and Dr. Mark McClellan. Dr. Gottlieb opened the May 4 meeting focusing on key agency priorities, such as the regulatory framework and use-reduction initiatives for tobacco products, modifications to food and nutrition labeling, and a new team-based approach

to product review at FDA’s Center for Drug Evaluation and Research.

Partnership, collaboration, and the role of the Reagan-Udall Foundation for the FDA were themes quick to emerge when the Commissioners sat down to discuss the Evolution of FDA Science and Engagement with moderator Susan Dentzer, President and CEO of the Network for Excellence in Health Innovation.



Each of the Commissioners highlighted the need for appropriate opportunities to share and learn more from academia and industry, recognizing that there are, as Dr. McClellan pointed out, “numerous science questions that FDA cannot answer by itself.” Dr. Gottlieb agreed, calling for transparent and thoughtful dialogue that maintains the independence of FDA.

The pace of scientific innovation is very quick, they noted, and regulatory science must adapt to where innovation is taking us to ensure a robust regulatory framework that supports physicians and patients. They also discussed the need to engage patients further in drug development and regulatory decision making and to implement strategies to move best practices from one scientific area to another with more accessible guidance.

Dr. Califf stressed the Foundation can help in initiating a policy dialogue among FDA, patients, consumers, developers, and others in the health ecosystem, noting that the Foundation’s role in “creating a venue where interaction can occur about the underlying science is critical.”

Drawing stakeholders together to explore challenges, validate approaches, and further scientific learning is a prime role the Foundation plays now and will continue to play into the future. Dr. von Eschenbach, who was commissioner at the Foundation’s start, reported that the Foundation grew out of FDA’s need to access critical intellectual capital beyond its walls.

The Commissioners lauded the Foundation for its work in expanded access and in post-market surveillance — especially highlighting the IMEDS contribution to real-world evidence.

Drawing stakeholders together to explore challenges, validate approaches, and further scientific learning is a prime role the Foundation plays.



➤ Advocates, industry representatives, and other attendees engage with the Commissioners



➤ Former FDA Commissioners Drs. McClellan, Califf, von Eschenbach and Gottlieb explore partnership, collaboration, and the role of the Foundation with moderator Susan Dentzer



➤ Attendees celebrate at the 2018 Innovations in Regulatory Science Awards Dinner



➤ Drs. Pazdur, O'Neill, and Tenaerts accept awards from Foundation leadership



2018

INNOVATIONS IN REGULATORY SCIENCE AWARDS

Celebrating the progress and the promise of regulatory science, the Foundation hosted its second [Innovations in Regulatory Science Awards](#) dinner on December 4, 2018. Two hundred people from government, academia, industry, and patient advocacy gathered to recognize the extraordinary contributions of the honorees in helping patients build better lives through regulatory science. The Foundation hopes that honoring today's change-makers will also inspire the next generation of researchers, innovators, and regulators.

➤ FDA Commissioner Gottlieb congratulates award winners



Honorees

Selected by an illustrious awards committee, Innovations in Regulatory Science Awards are presented in two categories: Leadership, honoring an individual's lifetime achievement and significant service in regulatory science and public health, and Innovation, recognizing an individual or organization that has made innovative contributions to regulatory issues, science, or policy.

The 2018 Leadership Award was presented to Dr. Richard Pazdur, recognizing his 20 years



of public service and his accomplishments as Director of FDA's Oncology Center of Excellence. Dr. Pazdur created an integrative approach to the clinical evaluation of drugs, biologics, and devices to treat cancer that now serves as the model for other diseases and future FDA centers of excellence. In his nomination Dr. Clifford Hudis of ASCO wrote that Dr. Pazdur "continues to work tirelessly to improve the efficiency of drug development and has challenged FDA staff to consider novel clinical endpoints and trial designs."

Two Innovation Awards were presented in 2018, recognizing both an individual and an organization for advancing regulatory science through groundbreaking initiatives.

Dr. Robert T. O'Neill, a leader in biostatistics who retired after 47 years of service to the FDA, advanced the use of quantitative methods in safety analysis. His work elevated clinical trial design and led to creation of the Office of Translational Sciences within FDA's Center for Drug Evaluation and Research. In making the award nomination, FDA's Dr. Robert Temple credits Dr. O'Neill with bringing "passion for doing something that would contribute to public health by using statistics."

Combining multi-stakeholder expertise with research findings, the Clinical Trials Transformation Initiative (CTTI) changed how the field approaches, designs, and conducts clinical trials. CTTI, a public-private partnership between Duke University and FDA, has used evidence-based approaches with 80 organizations to create more efficient, effective trials. Dr. Pamela Tenaerts, CTTI's Executive Director, says she believes the award "affirms that our efforts over the last decade have led to meaningful positive change in the quality and efficiency of clinical research."

Governance and Use of Funds

Twenty-four sponsorships — from patient advocacy and physician groups, biopharmaceutical companies, and non-FDA-regulated industry — combined with individual ticket sales to generate \$173,423. Johnson & Johnson was the presenting sponsor.

- Dr. Joanne Waldstreicher (left) of presenting sponsor Johnson & Johnson with Foundation Board member Kay Holcombe and 2017 Leadership honoree Dr. Janet Woodcock from CDER



Former Foundation Executive Director welcomes attendees



AWARDS COMMITTEE

Robert Califf, MD, MACC

Vice Chancellor for Health Data Science, Duke Health Advisor, Verily

William Chin, MD

Former Executive Vice President, Scientific and Regulatory Affairs, PhRMA

Garry Neil, MD — Committee Chair

Board Member, Reagan-Udall Foundation for the FDA; Chief Scientific Officer, Aevi Genomic Medicine

Patrick Ryan, PhD

Senior Director and Head of Epidemiology Analytics, Janssen Research and Development

Frank Sasinowski, MS, MPH, JD, FASCO

Director, Hyman, Phelps & McNamara

Richard Schilsky, MD, FACP, FASCO, FSCT

Board Vice Chair, Reagan-Udall Foundation for the FDA; Chief Medical Officer, American Society of Clinical Oncology

Janet Woodcock, MD

Director, FDA Center for Drug Evaluation and Research



- 200 people gathered to celebrate extraordinary contributions to regulatory science

FINANCIAL HIGHLIGHTS

The Reagan-Udall Foundation for the FDA's [audited financials](#) for the years ended December 31, 2018, and 2017:

REVENUE AND SUPPORT	2018	2017
FDA Transfer	\$1,250,000.00	\$1,250,000.00
Grants and Contributions	474,720.00	992,606.00
Contracts	573,125.00	-
Fundraising Event	133,607.00	62,877.00
Miscellaneous and Interest Income	9,172.00	5,831.00

Total Revenue and Support **2,440,624** **\$2,311,314**

EXPENSES AND CHANGES IN NET ASSETS	2018	2017
Program Services		
Critical Path to Tuberculosis Drug Regimens	-	15,813
Innovation in Medical Evidence Development and Surveillance	1,450,103	1,034,609
Expanded Access Navigator	111,158	346,966
Big Data For Patients	18,780	77,958

Total Program Services **\$1,580,041** **1,475,346**

Supporting Services		
Management and General	145,059	60,521
Fundraising	122,561	254,555

Total Supporting Services **267,620** **315,076**

TOTAL EXPENSES **\$1,847,661** **\$1,790,422**

CHANGE IN NET ASSETS	2018	2017
	\$592,963	\$520,892



Reagan-Udall Foundation for the FDA Leadership

The Reagan-Udall Foundation for the FDA could not support the mission of the Food and Drug Administration without the assistance of a diverse Board of Directors. The bi-partisan legislation that created the Foundation required that the board represent a cross-section of academic research, patient and consumer advocacy, health care providers, and regulated industry.

From left: Andrew von Eschenbach, Helen Darling, Ellen Sigal, June Wasser (former executive director), Scott Gottlieb (ex-officio), Allan Coukell, Richard Schilsky, and Kay Holcombe

Not pictured: Edward Allera, Georges Benjamin, Francis Collins (ex-officio), Sally Greenberg, Jonathan Leff, Mark McClellan, Garry Neil, Diana Zuckerman, and Lynn Zydowsky

2018 BOARD OF DIRECTORS

Ellen V. Sigal, PhD, *Board Chair*

Chair and Founder, Friends of Cancer Research. Dr. Sigal also serves on the Board of the Foundation for the National Institutes of Health.

Richard L. Schilsky, MD, *Vice Chair*

Chief Medical Officer, American Society of Clinical Oncology. Dr. Schilsky is an international expert in gastrointestinal malignancies and cancer pharmacology.

Helen Darling, *Treasurer*

Former interim President and CEO, National Quality Forum. Ms. Darling serves as Strategic Advisor for Health Benefits and Health Care in Washington D.C.

Kay Holcombe, *Secretary*

Former Senior Vice President for Science Policy, Biotechnology Innovation Organization. Ms. Holcombe also serves on the boards of the Critical Path Institute and the National Blood Clot Alliance.

Edward J. Allera, JD

Stakeholder and Co-Chair of the Food and Drug Practice at Buchanan Ingersoll & Rooney PC. Mr. Allera is both a pharmacist and an attorney with expertise in FDA regulation and biotechnology.

Georges C. Benjamin, MD

Executive Director, American Public Health Association. Dr. Benjamin is an expert on preventative healthcare.

Allan Coukell, BScPharm

Senior Director of Health Programs, Pew Charitable Trusts. Mr. Coukell oversees initiatives related to FDA, prescription drug spending, and drug and medical device innovation and safety.

Sally J. Greenberg, JD

Executive Director, National Consumers League. Ms. Greenberg is one of the board's consumer experts.

Jonathan Leff, MBA

Partner and Chairman, Deerfield Management. Mr. Leff also serves on the boards of the Spinal Muscular Atrophy Foundation and Friends of Cancer Research.

Mark McClellan, MD, PhD

18th Commissioner of the FDA. Dr. McClellan is the Director of the Duke-Margolis Center for Health Policy at Duke University.

Garry Neil, MD

Chief Scientific Officer, Aevi Genomic Medicine. Dr. Neil has held senior positions at Johnson & Johnson, Merck, and AstraZeneca.

Andrew C. von Eschenbach, MD

20th Commissioner of the FDA. Dr. von Eschenbach is President of Samaritan Health Initiatives, Inc., and a Senior Fellow at the Milken Institute.

Diana Zuckerman, PhD

President, National Center for Health Research. Dr. Zuckerman is a leader in patient advocacy.

Lynne Zydowsky, PhD

Co-founder/President of Alexandria Summit. Dr. Zydowsky is a biotechnology and life science executive who also serves as Chief Science Officer at Alexandria Real Estate Equities, Inc.

Scott Gottlieb, MD, *23rd FDA Commissioner (non-voting member)*

Dr. Gottlieb is a policy expert and public health advocate who previously served as FDA Deputy Commissioner for Medical and Scientific Affairs.

Francis Collins, MD, *NIH Director (non-voting member)*

Dr. Collins is known not only as NIH's Director but also for his leadership of the International Human Genome Project.

PARTNERSHIP *in* ACTION

In 2018, the Reagan-Udall Foundation for the FDA broadened and deepened its partnerships to advance regulatory science and its impact on America's public health. At the core of our work is our partnership with the U.S. Food and Drug Administration, which is made stronger and more effective by collaborating with the organizations and individuals recognized here.

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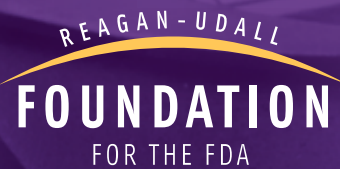


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