annual report | 2017

THE POWER *of* PARTNERING

REAGAN-UDALL FOUNDATION FOR THE FDA

REAGAN-UDALL

FOUNDATION

FOR THE FDA

Welcome Letter

New technology is creating “foundational opportunities” to treat and cure diseases, along with a concurrent need for new scientific tools to support the mission of the Food and Drug Administration (FDA). That was the message from FDA Commissioner Scott Gottlieb at the first-ever Innovations in Regulatory Science Awards in December 2017. And, he said, Congress created the Reagan-Udall Foundation for the FDA for precisely this purpose.

This year saw exciting progress at the Foundation in efforts to foster a better understanding of regulatory science, advance the use of real-world evidence for safety studies and help patients and the medical community navigate the process for accessing investigational drugs.

Supported by the Patient-Centered Outcomes Research Institute, our Big Data for Patients program developed workshop-ready tools for a community of empowered advocates.

The Innovation in Medical Evidence Development and Surveillance (IMEDS) program is creating a national resource for drug safety surveillance, building on the FDA’s Sentinel program.

We launched the Expanded Access Navigator to educate physicians and patients about access to investigational drugs and to help connect them with companies through a directory. The Navigator initially focused on oncology and is ready to serve broader patient needs, teaming next with the rare disease community.

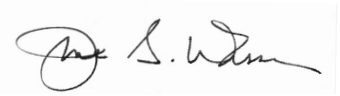
We are grateful for the many partners who have helped the Reagan-Udall Foundation for the FDA break new ground last year. We hope this update will inspire others to join us in supporting the mission of the FDA.

Ellen V. Sigal

June S. Wasser

Sincerely,

Ellen V. Sigal, Ph.D.
Board Chair



June S. Wasser, M.A.
Executive Director

Commissioner's Letter

Each day, patients and consumers across our country depend on FDA to fulfill a vital public health mission. In 2007, Congress created the Reagan-Udall Foundation to advance FDA's mission to not only modernize product development, but to accelerate innovation and enhance product safety. We are in the midst of a tremendously exciting era in medicine, with the potential of scientific advancements to transform our ability to diagnose, treat and cure diseases, and enhance our ability to confront the serious public health challenges we face, such as the tragic opioid crisis.

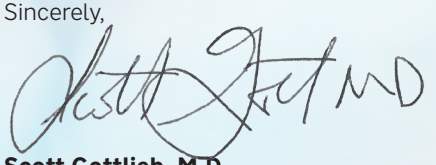
Scientific collaboration is critical to efficiently and effectively advancing our public health endeavors. We must be as modern as the products we are charged with regulating. Partnerships with organizations like the Reagan-Udall Foundation help FDA be better positioned to keep pace with scientific advancement, challenges, and opportunities — now and in the future. The Foundation embodies FDA's critical public health mission, and together, we've made significant strides in several key public health areas.

The Reagan-Udall Foundation has helped to foster and harness meaningful collaborations in furtherance of this vital public health mission. One example is our shared commitment to expanding access to safe and effective treatment options for patients, including those battling rare conditions and who often face unique medical challenges. Through the Foundation's unique statutory partnership and our collaborations with RUF, together we have widened the scope of the Navigator program to help maximize the opportunity for patients to access promising treatments through clinical trials when these treatments are otherwise unavailable.

Another successful outgrowth of our partnership with the Reagan-Udall Foundation is the IMEDS program, which serves as a national resource for broader public health and medical evidence generation, and is facilitating the use of real-world evidence generation and application in our regulatory decision-making.

These examples are just a glimpse into the potential for collaboration between FDA and the Reagan-Udall Foundation. I'm looking forward to building on the momentum of 2017 as we continue to work together to advance science, innovation and collaborations in pursuit of our vital patient and consumer mission.

Sincerely,



Scott Gottlieb, M.D.
FDA Commissioner and Ex-Officio Board Member of
the Reagan-Udall Foundation for the FDA

> **I believe that the creation of this foundation has made a difference in speeding up the development and evaluation of the safety of drugs being reviewed by the FDA and will make a real difference for those facing diseases with no cure today."**
>
> ~ U.S. Senator Michael B. Enzi

What's in a name? **A History of Partnering**

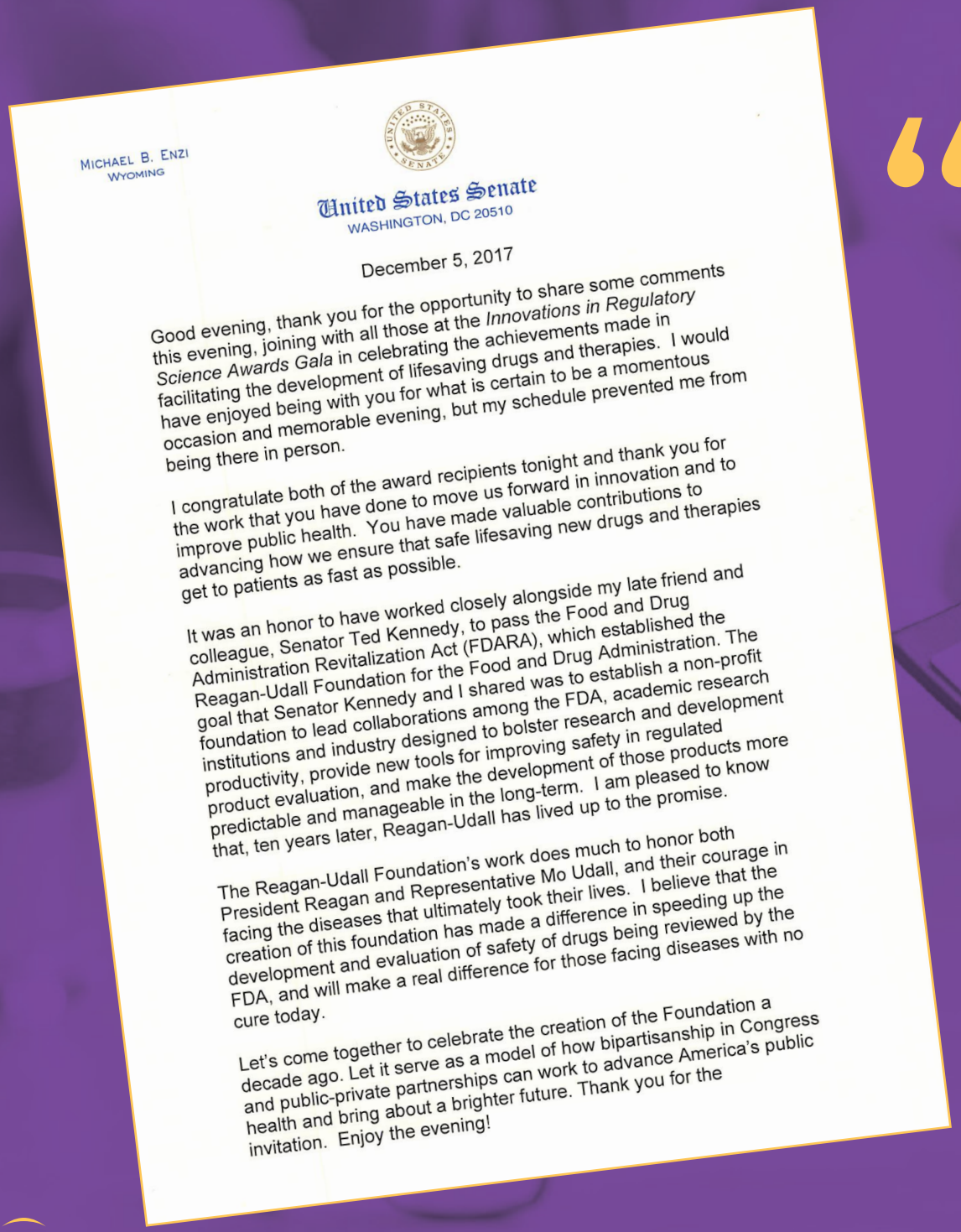
Using the power of partnering to meet modern public health challenges.

The Reagan-Udall Foundation for the Food and Drug Administration was named for Ronald Reagan, the 40th President of the United States, and Morris "Mo" Udall, the 14-term Democratic congressman from Arizona. Though politically different, the two had an important characteristic in common: both developed incurable neurodegenerative diseases. President Reagan died of complications from Alzheimer's and Congressman Udall battled Parkinson's for the last 18 years of his life.

Both leaders exemplified the imperative for developing treatments for unmet medical needs as well as new scientific tools to support FDA in its mission of evaluating new therapies. The FDA's Science Board formed a subcommittee of outside advisors in 2006, identifying collaboration as a key to meeting these needs.

Leading elected officials shared this belief in partnering to advance public health. Senators Ted Kennedy and Mike Enzi, the chair and ranking member, respectively, of the Health Education Labor and Pensions Committee, envisioned a nonprofit organization dedicated to building public-private partnerships as the place to start. The Food and Drug Administration Amendments Act, signed into law by President George H.W. Bush in 2007, established the Reagan-Udall Foundation for the FDA. The Foundation brings together representatives from FDA-regulated industries, academic experts, consumer groups and others — using the power of partnering to meet modern public health challenges.

Archival photo from July 22, 1981



MICHAEL B. ENZI
WYOMING

United States Senate
WASHINGTON, DC 20510

December 5, 2017

Good evening, thank you for the opportunity to share some comments this evening, joining with all those at the *Innovations in Regulatory Science Awards Gala* in celebrating the achievements made in facilitating the development of lifesaving drugs and therapies. I would have enjoyed being with you for what is certain to be a momentous occasion and memorable evening, but my schedule prevented me from being there in person.

I congratulate both of the award recipients tonight and thank you for the work that you have done to move us forward in innovation and to improve public health. You have made valuable contributions to advancing how we ensure that safe lifesaving new drugs and therapies get to patients as fast as possible.

It was an honor to have worked closely alongside my late friend and colleague, Senator Ted Kennedy, to pass the Food and Drug Administration Revitalization Act (FDARA), which established the Reagan-Udall Foundation for the Food and Drug Administration. The goal that Senator Kennedy and I shared was to establish a non-profit foundation to lead collaborations among the FDA, academic research institutions and industry designed to bolster research and development productivity, provide new tools for improving safety in regulated product evaluation, and make the development of those products more predictable and manageable in the long-term. I am pleased to know that, ten years later, Reagan-Udall has lived up to the promise.

The Reagan-Udall Foundation's work does much to honor both President Reagan and Representative Mo Udall, and their courage in facing the diseases that ultimately took their lives. I believe that the creation of this foundation has made a difference in speeding up the development and evaluation of safety of drugs being reviewed by the FDA, and will make a real difference for those facing diseases with no cure today.

Let's come together to celebrate the creation of the Foundation a decade ago. Let it serve as a model of how bipartisanship in Congress and public-private partnerships can work to advance America's public health and bring about a brighter future. Thank you for the invitation. Enjoy the evening!

> "Let it serve as a model of how bipartisanship in Congress and public-private partnerships can work to advance America's public health and bring about a brighter future."
>
> ~ U.S. Senator Michael B. Enzi

> Illustrative in highlighting real-world critical issues and provided a comprehensive understanding of how patient advocates can be involved in data sharing."
>
> ~ BD4P workshop participant

Big Data for Patients

In 2017, the Foundation concluded a two-year, Patient-Centered Outcomes Research Institute (PCORI) grant called Big Data for Patients (BD4P). "Big data" in medicine are patient data, and patient participation is vital to advancing patient-centered research initiatives. BD4P provided data science training to prepare patients and their advocates to participate in the design and evaluation of analyses that will affect their treatment options.

Goals

By equipping patients with tools to communicate about big data with policy makers, scientists, physicians and each other, BD4P's goal was to build a community of informed and empowered advocates.

Activities in 2017

The Foundation developed curriculum on scientific and ethical issues in the use of health data and disseminated training materials through workshops to patient advocates from 15 states. Attendees represented diverse disease interests, including cancer, asthma, allergies, multiple sclerosis, diabetes, mental health, gastrointestinal issues, epilepsy, Crohn's disease, heart disease, sickle cell disease and health disparity populations.

- Stakeholder Webinar on workshop development included a presentation by the National Cancer Institute's Office of Advocacy Relations
- Training Workshop

> **BD4P made me realize that there is actually quite a lot I can do as a patient advocate that involves the use of big data."**
>
> ~ BD4P workshop participant

> **"It provided a blue-print for how to take action as patient advocates"**
>
> ~ BD4P workshop participant

Big Data for Patients (continued)

- White Paper: *Exploration of the Development & Implementation of the Big Data for Patients (BD4P) Program* outlining best practices for adapting the training for different audiences or specific medical conditions
- Seven curriculum modules with prototypes for workshop development
- Final resources made available for further distribution and advocacy training

Governance and Use of Funds

Funding included a $250,000 Eugene Washington PCORI Engagement Award and $133,750 in other combined funding from Celgene Corporation, the Kaiser Institute for Health Policy, the Biotechnology Innovation Organization (BIO), the American Society of Clinical Oncology (ASCO) and PatientsLikeMe.

"Through our Big Data for Breast Cancer (BD4BC) initiative, Komen is working to develop and implement a strategy to unleash big data for the full benefit of breast cancer patients. One of the takeaways from our BD4BC meetings is patients and the public must be informed about big data and empowered to participate in big data-driven research. We commend you for the work you have done to introduce big data to patients; and are excited about this opportunity to continue this work and advance big data for breast cancer by raising patients' awareness, understanding and involvement."

~ Cheryl Jernigan, CPA, FACHE, Komen Advocate in Science and Scientific Advisory Board

Critical Path to Tuberculosis Drug Regimens

The Foundation ended work in 2017 on a $1,229,613 project support grant from the Bill and Melinda Gates Foundation called "Accelerating Critical Path to Tuberculosis Drug Regimens Work on Regulatory Science and Harmonization."

Goals

The Foundation's role was to increase awareness of the Critical Path to Tuberculosis Drug Regimens (CPTR) by providing leadership and program management support to two workgroups: Stakeholder & Community Engagement and Global Regulatory Pathways.

Specifically, the Foundation helped implement practice guidelines for TB drug trials and explored the potential for expediting the review of novel regimens in high TB-burden countries.

Activities

The Foundation worked to strengthen existing collaborations between organizations and individuals involved in community engagement. Workgroup activities supported by the Foundation included:

- 2014 *Evaluating the Impact of Community Engagement in TB Research session* at the 2014 Union Conference in Spain
- 2014 International Conference of Drug Regulatory Authorities in Brazil
- 2015 *Core Concepts and Practice* webinar
- 2016 workshop on the World Health Organization's collaborative procedure

Expanded Access Navigator

The Expanded Access Navigator represents a unique partnership among the Reagan-Udall Foundation for the FDA, physicians, patient advocacy organizations, the biopharmaceutical industry and the federal government to provide clear, digestible information on single-patient expanded access (EA). Launched in 2017, it's an online resource defining and explaining the process of requesting treatment use of medical therapies not yet approved by the FDA.

Background

The US Food and Drug Administration (FDA) has a long history of facilitating expanded access, often called "compassionate use," to enable patients with serious or life-threatening diseases to access medicines outside the context of clinical trials. Official regulatory pathways for EA have existed since 1987, and the Center for Drug Evaluation and Research (CDER) alone receives more than 1,000 EA requests yearly. The majority are for single patients, evenly split between emergency and nonemergency use, with 99.7% of all requests approved by the FDA. Despite this approval rate, more than 31 states have passed "Right-To-Try" legislation, fueled in part by the misperception that FDA authorization is a bottleneck.

DIA Conference, Chicago

FDA approval rate of expanded access requests

"When I was at FDA, we had a large staff answering questions from patients and physicians struggling to understand the process and figure out who to talk to, but our people could only manage the part of the process controlled by FDA, not the initial decision by companies," said Richard Moscicki, now Chief Medical Officer at the Pharmaceutical Research and Manufacturers of America (PhRMA). "As a neutral third party, the Reagan-Udall Foundation was the perfect vehicle to connect patients and physicians with sponsors conducting investigative analysis of new therapies."

"Nothing is more frustrating for a sick patient than not knowing who to call or sending random emails and never hearing back. What's great about the Navigator is not just that it provides contact information but also bite-sized pieces of content explaining the process and what happens."

~ Mark Fleury, Ph.D., Principal, Policy Development, American Cancer Society Cancer Action Network

NAVIGATOR'S OUTLINE OF EA PROCESS

PATIENTS use educational tools to work with...

PHYSICIANS who:

- Identify treatment
- Consider clinical trials
- (if patient can't join a trial)
- Ask pharmaceutical company for access to investigational therapy
- (if company authorizes)
- Submit request to FDA
- (if FDA approves)
- Send protocol to IRB
- (if IRB approves)
- Report adverse events and results to FDA

> **This is an extraordinary resource that is rich with helpful information. I'm thrilled that an effort has been made to empower physicians to navigate this complex area and to serve a previously unmet need in patient care."**
>
> ~ Dax Kurbegov, M.D., Vice President & Physician-in-Chief, Clinical Programs at Sarah Cannon, the Cancer Institute of HCA Healthcare

Navigation

Partnership

Education

Research

Outreach

Goals

The intent of the Navigator is to be a roadmap for single-patient expanded access requests. By increasing the availability and transparency of information, the Navigator aims to:

- Increase understanding of EA by filling information gaps and clarifying misconceptions
- Reduce the burden on physicians and patients of finding information on EA
- Increase willingness of physicians to explore EA for their patients
- Facilitate and expedite the request process
- Increase equity by ensuring that every patient, not just those with well-connected contacts or social media skills, is aware of EA as an option
- Improve physician and patient experience
- Assist FDA and industry in helping physicians better navigate the EA process

Achievements

The Foundation continued to build on scoping work initiated in 2016: an expanded access landscape review, stakeholder mapping and workshops, public comment solicitation and content and communication workgroups from industry, FDA, physician associations and patient advocacy organizations.

The successful launch of the Navigator in 2017 would not have been possible without partnering. Multiple webinars, conference presentations and one-on-one site demonstrations introduced the resource to targeted audiences for feedback before the national launch. The collaboration of communications experts across stakeholder groups extended the reach of traditional and trade media to early-adopters and influencers essential to long-term awareness and acceptance. Through them, patient and physician groups provided a pool of users to test the website's functionality. For example, a physician responding to "was this helpful?" prompts in user testing, said the resource was full of helpful resources he could have used early in his career.

JAN
- Project plan completed

APRIL
- Content reviewed by multi-stakeholder working group

MAY
- Website created
- Expanded Access Panel Discussion at Foundation Annual Public Meeting

Executive Director June Wasser at Indiana Life Sciences Summit

Rare Disease Stakeholder Meeting, Washington DC

"Clinical trials are the gold standard for showing that new therapies are safe and effective, and they offer individuals with rare diseases the opportunity to try innovative, and potentially life-improving, therapies. But for the vast majority of rare diseases, there is no clinical trial ongoing, and if there is, many patients may not qualify to participate. This is why we are excited to support the Navigator as it expands into rare diseases and helps rare disease patients navigate the process of obtaining investigational therapies."

~ Peter Saltonstall, President and CEO of the National Organization for Rare Disorders

Milestones

JUNE
Soft launch for user testing

JULY
Hard launch July 24, 2017, with national press release distribution and FDA Voice Blog Post

AUG
Partner outreach with Foundation-provided graphics and social media tools

SEPT
Improvements to directory functionality in September, resulting in higher industry participation

OCT
Expansion of Navigator from oncology focus to rare diseases requested by FDA, previewed in October congressional testimony

NOV
Rare Disease Navigator expansion exploration with stakeholders

DEC
Rare Disease Navigator expansion plan submitted to FDA in December — including recommendations on Guidances that would remove perceived barriers to biopharmaceutical company participation in the Navigator's Company Directory

2017 Results

- **$300,000** publicity value of earned media at launch
- **138** media mentions of Navigator since launch
- **62** million potential audience members reached
- **4,528** new users of the Navigator in 2017
- **6,809** unique page views of Navigator landing page
- **1,722** unique page views of the Company Directory
- **38** companies on Directory

Determining whether all approved options have been exhausted for patients with serious or life-threatening conditions can be very complicated for both physicians and patients. The Navigator guides them through this process, serving as a one-stop shop for expanded access programs from dozens of leading biopharmaceutical companies that also includes all the necessary forms, instructions, advocacy resources, and guides in a central location."

~ Clifford A. Hudis, M.D., FACP, FASCO
Chief Executive Officer, American Society of Clinical Oncology (ASCO)

Leading research-based companies want to make sure patients and physicians are not frustrated by the process. That's why they support, and want to partner with, the Navigator. The very number of patients that are now using it is a sign of progress."

~ Richard Moscicki, M.D., Chief Medical Officer and Executive Vice President, Science and Regulatory Advocacy at PhRMA

Expanded Access Navigator (continued)

Governance and Use of Funds

Fundraising for the launch of the initial, oncology-focused Navigator resulted in a total of $610,000 from a diverse group of committed stakeholders.

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

Though it is still too early to assess the impact of the Navigator on the quantity of EA requests that reach FDA, initial analyses by the Center for Drug Evaluation and Research (CDER) show an incremental increase in 2017.

Rising website traffic and positive reviews of the tool's usefulness are measures of the Navigator's early success. More importantly, though, they are indicators of the need to continue this important mode of educating and assisting physicians, patients, and caregivers. Expanding the Navigator will rely on the continuing partnership and sponsorship of many stakeholders. Clearly, though, early results demonstrate that the Navigator is an essential patient-focused resource.

"THIS PROGRAM IS LITERALLY A LIFELINE FOR PATIENTS WHO HAVE TRIED TREATMENT AFTER TREATMENT, AND SUDDENLY FIND THEMSELVES OUT OF OPTIONS."

NAVIGATOR.REAGANUDALL.ORG

> **Having a neutral convening entity is important to enable stakeholders to collaborate on the best and most appropriate use of real-world evidence to support decision making. And when a sponsor comes to IMEDS they know they're promising to make the results public and form part of the knowledge base informing how care should be provided to patients."**
>
> ~ Jeff Brown, Ph.D., Director of IMEDS Analytic Center at Harvard Pilgrim Health Care Institute

Innovation in Medical Evidence Development and Surveillance (IMEDS)

This public-private partnership, modeled after the FDA's Sentinel Initiative, provides industry and other researchers access to large databases of de-identified healthcare claims data and FDA-validated analytic tools and methods used to study the safety of marketed medical products. Study sponsors work with the Foundation, Sentinel data partners and the analytic center at Harvard Pilgrim Health Care Institute to answer questions of public health interest.

Background

The Sentinel Initiative fulfills FDA's congressional mandate to develop a distributed database composed of insurer claims information for products already on the market. The initial vision was for Sentinel to be available to researchers outside of the Agency and IMEDS fulfills this goal.

In a video created for the Reagan-Udall Foundation website, Dr. Janet Woodcock, Director of the FDA's Center for Drug Evaluation and Research, enumerated future applications for IMEDS beyond those of industry fulfilling regulatory obligations like safety signal refinement, post-market safety studies and Risk Evaluation and Mitigation Strategies. These include cohort identification for clinical trials and drug utilization including combination therapy and additional indications. IMEDS can also compare outcomes among different patient groups using different drugs.

Goals

While there are other pathways to respond to safety signals or conduct post-market research, IMEDS provides a single entry point to healthcare data on millions of patients, in a secure environment that protects patient privacy. In many cases, because of the size of the database, this approach could provide answers to previously unanswerable questions.

We selected the IMEDS data for two reasons: the first was size. Since we were looking at a rare outcome we needed a large data source. The second reason was the availability of validated tools for conducting the query… Anybody starting with your parameters is going to get exactly the same results so that's very reassuring both to industry and I'm sure to regulators."

~ Dr. Claudia Salinas, Senior Research Scientist,
Global Patient Safety Epidemiology at Eli Lilly and Company

IMEDS (continued)

In his 2017 FDA Voice blog post introducing IMEDS, then-Commissioner Robert M. Califf, M.D., said IMEDS shifts the focus from debates over differing methods and data to the underlying clinical and public health questions of concern. "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."

Public health matters that IMEDS could address include characterizing hard-to-reach populations, like rare conditions and mother-child linkages, reducing the time between a safety signal and full evaluation and customizing epidemiological studies.

The benefits of IMEDS go beyond the goals of individual research sponsors. Through the collaborative nature of the program and researchers' agreement to publish results, participants contribute to the growing body of safety-surveillance tools and methods.

The benefits of IMEDS go beyond the goals of individual research sponsors.

Marcus Wilson, the Chair of the IMEDS steering committee and President of HealthCore, Inc., an IMEDS data partner research organization, calls the program a conduit through which insight is generated and shared.

More precise decision-making tools can be developed by leveraging evidence generated by the IMEDS framework. This could change everything — from how a product gets labeled by FDA to how doctors determine the benefit/risk profile for patients they treat."

~ Marcus Wilson, Pharm.D.,
Co-Founder & President
HealthCore, Inc.

Active surveillance through a **large volume of patient data** via private and secure network

Accepted methods and tools relied upon by FDA using the Sentinel Common Data Model

Ensures transparency of operations, methods and publications

Achievements

IMEDS successfully transitioned from a pilot study in 2015 and three grants for academic methods research in 2016, to ongoing, industry-funded studies in 2017.

Results of the IMEDS pilot study were published in *Pharmacoepidemiology and Drug Safety* in March of 2018. The Pfizer-sponsored pilot was the first to test whether stakeholders other than FDA could successfully direct scientific queries using IMEDS. Specifically, the study evaluated a class-wide label change for proton pump inhibitors, which are used in the prevention and treatment of ulcers and acid-related conditions such as gastroesophageal reflux disease. Because of a finding that the label change for proton pump inhibitors may have influenced subsequent prescribing behaviors, the study concluded that large, distributed health data networks can be used to assess the effectiveness of risk minimization.

The Foundation partially funded a Sentinel-related methods paper, also published in 2018 in *Pharmacoepidemiology and Drug Safety,* which discusses key issues for using sequential surveillance in a distributed database of electronic health records, and when this can contribute to regulatory decisions.

In 2017, Novartis started a collaboration with IMEDS for a feasibility analysis in the context of an epidemiological study. And Eli Lilly and Company launched an IMEDS study to provide context about the safety profile of patients with rheumatoid arthritis. It looked at patients treated with disease modifying anti-rheumatic drugs, to estimate the incidence of venous thromboembolism. The study results were included as part of Lilly's resubmission for baricitinib and shared at the 2018 Sentinel Public Meeting.

IMEDS potential uses include data on mother-child linkages

2017 RESULTS

35 companies requested IMEDS educational materials

17 companies participated in IMEDS webinars

16 companies participated in two IMEDS workshops

16 use case discussions were held with companies

2 projects have been completed

> **"It was as a result of the strong partnership of the team that we were able to get our results and incorporate them into our document…Disclosing the results of the query with the broader community of rheumatoid arthritis researchers and clinicians was a win-win for both of us."**
>
> ~ Dr. Claudia Salinas, Senior Research Scientist, Global Patient Safety Epidemiology at Eli Lilly and Company

Provides timely and efficient processes for evaluation analyses

Collaborative partnership with public and private sector organizations including healthcare payers, academic institutions, pharmaceutical companies and the FDA

Leveraging and contributing to collective knowledge accumulated by multiple stakeholders over years of routine use for real world evidence generation

TYPES OF DATA USED IN IMEDS

- Office Visit Diagnostic Code
- Prescription Dispensing Code
- Emergency Department Procedure
- Inpatient Hospital Stay

=

Sources of Exposure and Outcomes Data

> **Developing methodology around important health outcomes, for example stroke, heart attack or seizures, will allow FDA to use the same algorithms. Reagan-Udall is well positioned for this type of collaboration because the data has so much complexity and subtlety."**
>
> ~ Jeff Brown, Ph.D., Director of IMEDS Analytic Center at Harvard Pilgrim Health Care Institute

IMEDS (continued)

Governance and Use of Funds

The Foundation reviews and facilitates all IMEDS studies, provides scientific and business management oversight and secures Institutional Review Board approval. As the neutral convening party, it ensures transparency of operations, methods and publications resulting from IMEDS projects. Industry scientists are essential study team members — from protocol development through implementation.

The combined, prior-year committed funds and new funds raised in 2017 provided $1,355,400 in financial support and funded research.

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

The Foundation began work on a project called "IMEDS: Validation of Health Outcomes, Exposures, and Cohorts of Interest" in 2017. This will bring together multiple industry sponsors and the IMEDS data and analytic partners to validate algorithms using the 10th revision of the International Classification of Diseases (ICD-10) codes.

Compared to its predecessor, ICD-9, the current classification dramatically increases the number of possible medical diagnosis codes used by health care professionals, hospitals, insurers and other payers and sets the stage for improved patient care and public health surveillance. Stakeholders agree that careful mapping from ICD-9 to ICD-10 and validation studies across prioritized health outcomes are critical. Through collaborative work and use of a robust distributed database, this IMEDS project will validate algorithms to share with FDA and the entire research community.

Reagan-Udall Foundation Fellowships at the FDA

Another program making the transition from planning to partnering in 2017 was the Foundation's fellowship program: a unique and hands-on experience for post-doctoral and other career scientists in new regulatory pathways, modernizing clinical trial design and expediting the review of new medical products.

One of FDA's 2018 priorities is strengthening the scientific workforce

The legislation creating the Foundation called for training scientists, doctors and other professionals to foster greater understanding of, and expertise in, new scientific tools, diagnostics, manufacturing techniques and potential barriers to translating basic research into clinical and regulatory practice.

One of FDA's 2018 priorities is strengthening the science workforce, and Denise Hinton, the agency's Acting Chief Scientist, sees the Foundation fellowship as a potential pipeline for expertise.

Goals

The intent of the fellowship program is to train scientists for post-fellowship employment by providing them with unique experience in regulatory challenges such as those addressed in the 21st Century Cures Act. Examples of possible research topics include epidemiology, biostatistics, bioinformatics, comparative effectiveness research, clinical trial design and the use of "big data" in real-world evidence generation.

Achievements

The Foundation developed a Memorandum of Understanding with the FDA, detailing roles and responsibilities, and formed a Joint Steering Committee for planning, implementation and evaluation of the program.

Governance and Use of Funds

Curriculum development, candidate selection, research project approvals and evaluation will be collaborative, while the Foundation takes the lead role in securing underwriters and managing the program. Exploratory outreach to potential funders in 2017 gathered perspectives on career opportunities to meet the specialized technical demands of the future.

> **Unlike other FDA Fellowship Programs that focus solely on work at FDA, the Foundation Fellows gain a broad perspective of public health. In addition to FDA's regulatory process, they learn how academia and industry participate in medical product research and development."**
>
> ~ RADM Denise Hinton, FDA Acting Chief Scientist

Awards Committee

Robert Califf, M.D.
22nd Commissioner of the FDA

Garry Neil, M.D.
Chief Scientific Officer,
Aevi Genomic Medicine

Richard Schilsky, M.D.
Senior Vice President and
Chief Medical Officer, ASCO

William Sellers, M.D.
Core Faculty Member, Broad Institute,
Dana-Farber Cancer Institute and
Harvard Medical School

Robert Temple, M.D.
Deputy Center Director for Clinical
Science, CDER, and Acting Deputy
Director of the Office of
Drug Evaluation

FDA Commissioner Scott Gottlieb
giving opening remarks

Board member Georges Benjamin
reading remarks by Senator Mike Enzi

Healthcare Innovation Awardee Patrick Ryan
(Janssen Research and Development) with
Leadership Awardee Janet Woodcock (FDA)

Innovations in Regulatory Science Awards

Behind every breakthrough in public health are regulators, scientists, data analysts and policy makers dedicated to safety, efficacy and accountability. The Innovations in Regulatory Science Awards, developed by the Foundation and first awarded at a dinner event in December, 2017, aim to raise public awareness of innovation in regulatory science and inspire the next generation. A wide range of sponsors supported the event, which was intended both to recognize the achievements of the awardees and raise funds and awareness for future Foundation activities.

The Foundation convened a distinguished awards committee that included leading representatives of government, industry, physician advocacy and academia.

Awardees

For the inaugural year, the Awards Committee focused on two categories: Leadership, recognizing an individual with significant lifetime contributions and service to regulatory science and public health, and Healthcare Innovation, honoring either an individual or organization advancing regulatory science through novel initiatives.

The Director of FDA's Center for Drug Evaluation and Research, Dr. Janet Woodcock, received the Leadership Award. During her 30 years at the FDA, Dr. Woodcock has contributed to advances in pharmacogenomics and the regulation of novel therapies arising from biotechnology, as well as initiatives to ensure pharmaceutical risk management, data standardization and drug safety.

The recipient of the Healthcare Innovation award, Dr. Patrick Ryan, is the Sr. Director and Head of Epidemiology Analytics at Janssen Research and Development. He led efforts developing data collection alternatives to traditional randomized trials through the Observational Health Data Sciences and Informatics network (OHDSI).

Governance and Use of Funds

Individual ticket sales and sponsorships from the following groups raised $90,400.

FDA's Anna Abram with Reagan-Udall Foundation Board Member Allan Coukell and Presenting Sponsor Deloitte's Asif Dhar

Presenting Sponsor

Deloitte.

Sponsors at the "Innovations" level

Johnson & Johnson OFFICE OF THE CMO

PhRMA RESEARCH • PROGRESS • HOPE

Sponsors at the "Regulatory" level

Lilly

Greenleaf Health

Sponsors at the "VIP" level

Amicus Therapeutics
Aevi Genomic Medicine
Alston & Bird
American Society of Clinical Oncology (ASCO)
Biotechnology Innovation Organization (BIO)
Booz Allen Hamilton
Flagship Pioneering
Grocery Manufacturers Association (GMA)
Pepper Hamilton LLP
Susan G. Komen
and venue donated by Kaiser Permanente

PhRMA's Bill Chin and Steve Ubl with NIH Director Francis Collins

Attendees at inaugural Innovations in Regulatory Science Awards

A five-member host committee provided direction and encouraged a broad range of support for the inaugural event

Host Committee

Louis DeGennaro, Ph.D.
President and CEO,
Leukemia and Lymphoma Society

Richard Gaynor, M.D.
President of Research and Development,
Neon Therapeutics

Julie Hambleton, M.D.
Strategic Consultant, former Vice President and Head of U.S. Medical Affairs,
Bristol-Meyers Squibb

Margaret Hamburg, M.D.
21st Commissioner of the FDA

Lynn Matrisian, Ph.D.
Chief Science Officer,
Pancreatic Cancer Action Network

Financial Highlights

The Reagan-Udall Foundation for the FDA's audited financials for the years ended December 31, 2017, and 2016:

REVENUE AND SUPPORT	2017	2016
FDA Transfer	$1,250,000	$1,000,000
Grants and Contributions	992,606	2,669,596
Fundraising Event	90,400	–
Miscellaneous and Interest Income	5,831	11
Total Revenue and Support	**$2,338,837**	**$3,669,607**

EXPENSES AND CHANGES IN NET ASSETS	2017	2016
Program Services		
Critical Path to Tuberculosis Drug Regimens	$15,813	$141,151
Innovation in Medical Evidence Development and Surveillance	1,034,609	1,910,483
Expanded Access Navigator	346,966	95,030
Big Data For Patients	77,958	250,076
Total Program Services	**$1,475,346**	**$2,396,740**
Supporting Services		
Management and General	282,078	188,986
Fundraising	60,521	19,740
Total Supporting Services	**342,599**	**208,726**
TOTAL EXPENSES	**$1,817,945**	**$2,605,466**

CHANGE IN NET ASSETS	2017	2016
	$520,892	$1,064,141

Reagan-Udall Foundation for the FDA Board members

The Reagan-Udall Foundation 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.

Back row, left to right: Jonathan Leff, Allan Coukell, Garry Neil and Georges Benjamin.
Front row, left to right: Helen Darling, Richard Schilsky, Ellen Sigal and Kay Holcombe.
Not pictured: Mark McClellan, Pamela Bailey, Sally Greenberg, Diana Zuckerman and Ex-Officio members.

Board of Directors

Ellen V. Sigal, Ph.D., 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, M.D., 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.

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

Pamela G. Bailey
President and CEO, Grocery Manufacturers Association. Ms. Bailey brings food and cosmetic industry expertise to the Foundation's board.

Georges C. Benjamin, M.D.
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 generally, prescription drug spending and drug and medical device innovation and safety.

Sally J. Greenberg, J.D.
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.

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

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

Scott Gottlieb, M.D., 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, M.D., 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.

Our Partners

In 2017, the Foundation spearheaded complex research collaborations detailed in the pages of this report. The measurable progress made demonstrates the power of partnering, and the logos shown here represent those who helped the Foundation advance regulatory science in new ways.

Critical Path to Tuberculosis Regimens

Bill & Melinda Gates foundation

Big Data for Patients

ASCO® American Society of Clinical Oncology

Bio Biotechnology Innovation Organization

Celgene

KAISER PERMANENTE®

pcori® Patient-Centered Outcomes Research Institute

patientslikeme®

Bio Biotechnology Innovation Organization

FOUNDATION MEDICINE®

janssen Pharmaceutical Companies of Johnson & Johnson

Pfizer

PhRMA Research • Progress • Hope

MERCK INVENTING FOR LIFE

Expanded Access Navigator

American Cancer Society®

ASCO®
AMERICAN SOCIETY OF CLINICAL ONCOLOGY

Bristol-Myers Squibb

Genentech
A Member of the Roche Group

Lilly

SUSAN G. KOMEN®

Innovations in Medical Evidence Development and Surveillance

aetna®

AstraZeneca

Harvard Pilgrim HealthCare

HealthCore®

HealthPartners® Institute

Comprehensive Health Insights℠
A Humana Company

Lilly

Marshfield Clinic®
Research Institute

MERCK
INVENTING FOR LIFE

The Meyers Primary Care Institute

NOVARTIS

Pfizer

VANDERBILT UNIVERSITY
MEDICAL CENTER

REAGAN-UDALL
FOUNDATION
FOR THE FDA

To join the Reagan-Udall Foundation for the FDA in improving public health and advancing regulatory science, please visit our website at **www.reaganudall.org** or contact the Foundation directly at **202.849.2075**

The Reagan-Udall Foundation for the FDA is an independent 501 (c) (3) created by Congress.

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