# 2024 ANNUAL REPORT

Advancing the mission of the FDA to modernize product development, accelerate innovation, and enhance product safety

REAGAN-UDALL FOUNDATION FOR THE FDA

# About Us

The Reagan-Udall Foundation for the Food and Drug Administration is an independent 501(c)(3) organization created by Congress "to advance the mission of the FDA to modernize medical, veterinary, food, food ingredient, and cosmetic product development, accelerate innovation, and enhance product safety."

The Foundation embodies the FDA's vision of collaborative innovation to address regulatory science challenges of the 21st century and assist in the creation of new, applied scientific knowledge, tools, standards, and approaches the FDA needs to evaluate products more effectively, predictably, and efficiently, and thereby enhance the FDA's ability to protect and promote the health of the American public. The Foundation serves as a crucial conduit between the FDA and the public, providing a means for the FDA to interact directly with stakeholders, including industry and consumers. The Foundation does not participate in regulatory decision-making or offer advice to the FDA on policy matters.

REAGAN-UDALL FOUNDATION FOR THE FDA

# Foundation Leadership Message

The Reagan-Udall Foundation for the FDA plays a vital role in supporting the FDA's commitment to ensuring the health of all Americans. In 2024, we helped advance the Agency's mission by propelling innovation, transparency, and efficiency in regulatory science.

Our focus on patients and consumers provides a constant reminder of the FDA's real-world impact, and the feedback we offer the Agency adds important context that supports decision-making. In 2024, we engaged with the rare disease community to inform the Agency's newly launched Innovation Hub and to explore the roles of biomarkers and natural history data in understanding the biology and clinical manifestations of rare diseases. We also increased the reach of the Expanded Access Navigator, a valuable tool for doctors and patients to find investigative treatments. Patient listening sessions on a variety of topics yielded rich input to inform clinical trial design, product development, and life-cycle management.

Ensuring that Americans have access to safe, nutritious food is a high priority, especially in the face of an epidemic of chronic disease. With an expanded food portfolio, we facilitated dialogue among participants in the food supply chain to better understand needs, available data, and the challenges they face in monitoring and improving access to safe and nutritious foods.

Through our long-running Innovation in Medical Evidence and Development Surveillance (IMEDS) program, we managed robust post-market studies that leverage FDA tools to better understand the frequency and severity of specific adverse events and to help identify high-risk populations. To modernize post-market evidence generation, we held public webinars on real-world evidence and led projects to improve the evaluation of data sources and the use of algorithms to evaluate real-world data.

The high prevalence of substance use disorder demanded our attention on issues such as online availability of controlled substances. We hosted discussions exploring the risks and potential benefits of drugs such as ketamine and psychedelics. And as the need for mental health treatment continued to grow, we strategized with researchers, clinical experts, and patients about promising digital tools to assist disease evaluation and management.

As 2025 began, we welcomed new leadership at the FDA. We look forward to working together in continued pursuit of innovation and reform to both protect and promote the public's health. We are grateful to the Agency and the many other collaborators who engage in our vital work.

Sincerely,

**Susan C. Winckler, RPh, Esq.**
Chief Executive Officer
Reagan-Udall Foundation for the FDA

**Richard L. Schilsky, MD, FACP, FSCT, FASCO**
Chair, Board of Directors
Reagan-Udall Foundation for the FDA

> To advance the mission of the FDA, the Foundation prioritizes the value of science and rigorous data to solving the challenges of public health.
>
> Commissioner Martin A. Makary



# FDA Commissioner Message

The FDA owes it to all Americans to keep pace with scientific innovation while rigorously ensuring the safety and quality of the food and medical products we regulate. As part of an administration dedicated to making America healthy, I look forward to the continuing assistance and collaboration of the Reagan-Udall Foundation for the FDA.

To advance the mission of the FDA, the Foundation prioritizes the value of science and rigorous data to solving the challenges of public health.

The Foundation provides the Agency with crucial analysis and linkages across the health ecosystem on topics such as rare disease treatments, post-market research, substance use, and food data. These contributions help the FDA do more to address the challenges we face, from chronic diseases to counterfeit prescription drugs.

Patient needs are a central driver in our decision-making, so it is critical that we hear from consumers and advocates. The Foundation provides a platform for direct, transparent engagement through listening sessions and public discourse. Similarly, the Foundation connects the FDA to innovators from multiple sectors to streamline regulatory pathways, enabling the public to benefit from pioneering discoveries.

More than ever, the FDA is called upon to empower consumers to make healthy choices they can have confidence in. The Foundation's work and ability to adapt to a complex, rapidly changing health landscape bolsters the FDA's ability to fulfill its mission. I extend my gratitude to the Foundation for its instrumental support as we move forward with a renewed focus on progress, transparency, and common sense.

Sincerely,

**Martin A. Makary, MD, MPH**
Commissioner of Food and Drugs
Food and Drug Administration

# Rare Diseases

Rare diseases impact more than 400 million people worldwide, including nearly 30 million Americans — half of them children. The vast majority of rare diseases have no FDA-approved treatment available.

## Advancing Rare Disease Therapies Through an FDA Rare Disease Innovation Hub

As the FDA announced the Rare Disease Innovation Hub, they turned to the Foundation to bring the rare disease community together to inform the priorities of this new effort to spur treatment development.

Agency leaders envisioned the Hub as a model for enhancing cross-center collaboration on rare diseases, especially between the Center for Biologics Evaluation and Research and the Center for Drug Evaluation and Research. They recognized the imperative for efficient, agile regulatory frameworks that evolve with innovation — without compromising safety. Woven throughout the public meeting was a commitment to engaging rare disease patients and addressing their unique needs to overcome challenges at every stage of therapeutic development. From Agency discussion to the 37 public comments presented, 15 key themes emerged, including prioritizing external communication between the FDA and the rare disease community, building transparency in decision making, and revolutionizing clinical trials.

*This project was supported through a cooperative agreement with the Food and Drug Administration.*


Public comment session at the FDA Rare Disease Innovation Hub meeting in fall 2024

FROM LEFT: Susan C. Winckler, RPh, Esq., Reagan-Udall Foundation for the FDA; Kerry Jo Lee, MD, FDA; Julie Tierney, JD, FDA

### Advancing Regulatory Science

Explored biomarkers, endpoints, and natural history studies to improve meaningful patient outcomes

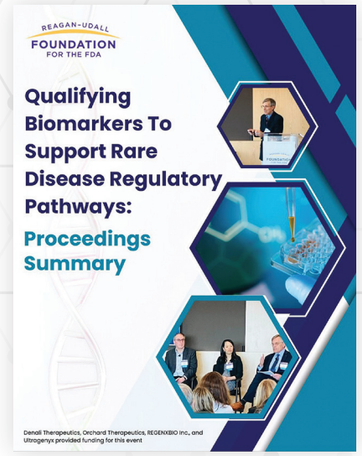
### Disseminating Information

Shared findings and innovative strategies through 4 meeting summaries and reports

### Facilitating Engagement

Convened more than 4,500 participants in public meetings on rare diseases






## Qualifying Biomarkers to Support Rare Disease Regulatory Pathways

In early 2024, the Foundation held a hybrid public workshop on the application of biomarkers to speed approval of treatments for rare diseases, a topic that offers hope to patients.

Biomarkers are measurable indicators of disease progression or treatment response that can provide early, reliable predictors of outcomes. Crucially for rare diseases, biomarkers can help shorten clinical trials, but they require broad, rigorous evaluation.

As a case study, workshop speakers explored the biomarker heparan sulfate in neuronopathic mucopolysaccharidoses (MPS), inherited metabolic diseases that pit families in a race against time to limit progressive disability in their children.

Patient advocates, researchers, regulators, and industry representatives engaged in a dialogue on challenges and potential solutions. They emphasized the need to combine data and improve collaboration across various stages and sources, including basic research, natural history studies, animal studies, and clinical trials.

*This project was supported by Denali Therapeutics, Orchard Therapeutics, REGENXBIO, and Ultragenyx.*

> Our regulatory system must evolve to reflect advancing science. That is precisely the reason we are leaning into tools like biomarkers … (that) can move the needle in delivering novel therapies to patients in need.

Gavin Imperato, MD, PhD, Center for Biologics Evaluation and Research, FDA


FROM LEFT: John Crowley, JD, MBA, Amicus Therapeutics, Biotechnology Innovation Organization; Cara O'Neill, MD, Cure Sanfilippo Foundation; Cherie Fathy, MD, MPH, FDA; Susan C. Winckler, RPh, Esq., Reagan-Udall Foundation for the FDA; Edward Neilan, MD, PhD, National Organization for Rare Disorders; Carole Ho, MD, Denali Therapeutics; James Wilson, MD, PhD, University of Pennsylvania; Gavin Imperato, MD, PhD, FDA (virtual)

FROM LEFT: Benjamin Forred, MBA, ACRP-CP, Sanford Research; Tiina Urv, PhD, NIH; Donna Rivera, PharmD, MSc, FDA; Kimberly Smith, MD, MS, FDA

### Natural History Studies and Registries in the Development of Rare Disease Treatments

Finding effective treatments for a disease requires understanding how the disease progresses over time — its natural history. For many rare diseases, the natural history is poorly understood because few patients are diagnosed and studied in an organized way.

In partnership with the FDA's Center for Drug Evaluation and Research and the National Institutes of Health National Center for Advancing Translational Sciences, the Foundation convened a hybrid public workshop on collecting and leveraging robust, standardized natural history data to improve and inform development of rare disease treatments.

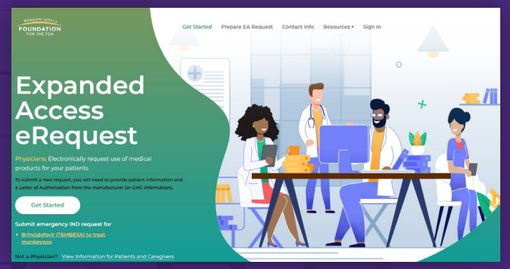
Presentations identified potential solutions to funding, ethics, data sharing, and privacy challenges. Speakers also shared lessons from state-of-the-art initiatives that have informed regulatory decision-making across a range of rare diseases — including progeria, porphyrias, Friedreich's ataxia, and hyperoxaluria — with meaningful benefit for patients.

> "Stakeholder engagement … is absolutely critical. You need to understand your key stakeholders and engage them all early and often throughout a patient registry process."
>
> Kristen Wheeden, MBA, United Porphyrias Association

*These projects were supported through a cooperative agreement with the Food and Drug Administration.*

## EXPANDED ACCESS NAVIGATOR

When seriously ill patients have exhausted their treatment options, they often do not know where to turn. The Foundation's online Expanded Access Navigator provides step-by-step guidance to help patients, caregivers, and health care providers explore, and often obtain, investigational treatments. Expanded access, or compassionate use, is particularly important for patients who do not qualify for clinical trials or may not live near trial sites.




This useful and trusted program for empowering patients continued to grow in 2024, with more than half of all expanded access requests submitted to the FDA through the Navigator's eRequest app. The Navigator web resource saw a 20 percent increase in visits, and the number of drug developers listing their resources in the Navigator directory increased by nearly 25 percent.



FROM LEFT: Cecelia Calhoun, MD, MPHS, MBA, Yale University School of Medicine; Julie Makani, MD, PhD, Muhimbili University of Health and Allied Sciences, Tanzania High Commission to the UK; Susan C. Winckler, RPh, Esq., Reagan-Udall Foundation for the FDA; Peter Marks, MD, PhD, FDA; Kwasi Nyarko, PhD, World Health Organization Regional Office for Africa; Jimi Olaghere, gene therapy recipient; Hildegard Büning, PhD, Hannover Medical School; Jeremy Farrar, MD, PhD, World Health Organization (virtual)

## Gene Therapies

### Global Opportunities in Gene Therapies

The FDA has approved pioneering gene therapies to treat diseases such as sickle cell, thalassemia, hemophilia, and Duchenne's muscular dystrophy, and more therapies are in the pipeline. How can global regulators help bring these lifesaving discoveries to patients worldwide?

In September 2024, the Foundation gathered international experts to explore opportunities for regulatory convergence in emerging markets. During the public workshop, advocates, clinicians, regulators, academic researchers, and industry representatives highlighted concerns such as cost and ethics, as well as critical themes for international collaboration:

- Bidirectional learning
- Patient-centered approaches
- Investment in local capacity and regional infrastructure
- Long-term follow-up
- Strategies to reduce costs

Ultimately, a holistic approach is needed to make current and next generation gene therapies affordable, accessible, and sustainable in regions with limited resources.

*This project was hosted in collaboration with the Bill and Melinda Gates Foundation.*

> "We need to find more options and give patients more time, another day to fight … I would love for this to be the last generation of sickle cell as we know it."
>
> Jimi Olaghere, gene therapy recipient

# Food and Nutrition

Chronic diseases related to diet — such as cardiovascular disease, diabetes, and certain cancers — account for more than 1 million American deaths each year.

## Real-world Data to Assess Long-term Impact of FDA Food-Related Regulations and Policies: A Snapshot

At the FDA's request, the Foundation investigated the availability of baseline data and analyses from across the food supply chain, guided by an expert working group and informed by interviews with 27 ingredient and product manufacturers, distributors, food service providers, and retailers.

The data landscape that emerged is a complicated one. Retail nutrition data are available but are costly and inconsistent, and food service outlets like restaurants lack comparable data systems. Approaches to food safety data are reactive to outbreaks but miss opportunities for prevention.

Public health and the economy rely on food that is safe and nutritious, but we found that current data practices do not generate the robust evidence needed to measure the impact of food regulations. Opportunities to fill the gaps include creating partnerships that incentivize data sharing, establishing a digitized food ecosystem, and leveraging technology to improve data capture and analysis. Findings from the report were presented at the Institute of Food Technologists conference in July 2024.


> "In an era of broader transparency, all sectors are challenged to elevate the accessibility of data — especially with the nation's health outcomes at stake."
>
> Snapshot report

*The projects in the Food and Nutrition portfolio were supported through a cooperative agreement with the Food and Drug Administration.*

**Advancing Regulatory Science**

Reported on data needs and operational changes to improve food safety and nutrition regulation

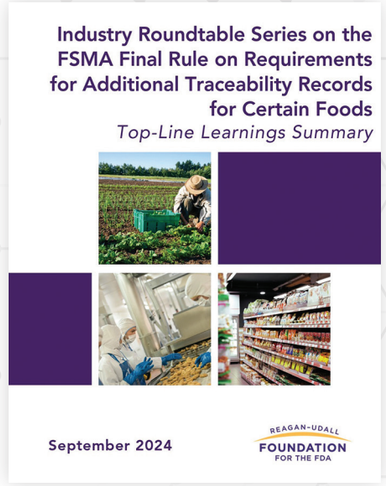
**Disseminating Information**

Activated a Nutrition Communications Network to share science-based nutrition messages

**Facilitating Engagement**

Worked across the food supply chain with 90 experts and nearly 600 public meeting participants






## Industry Roundtable Series on the FSMA Final Rule on Requirements for Additional Traceability Records for Certain Foods

The Food Safety Modernization Act (FSMA) calls for enhanced record-keeping by producers along the food supply chain. The changes aim to fast-track the identification and removal of contaminated foods, reducing foodborne illness and death.

At the FDA's request, the Foundation convened three food industry roundtables to gather perspectives on implementation of the Act's Food Traceability Final Rule. The discussions among 34 individuals from across the farm-to-table continuum highlighted concerns such as low awareness and readiness, complexities of traceability lot codes and labeling, and capabilities of warehouse management systems. Potential strategies for success include education and best practices, pilot programs, a staggered implementation schedule, and a public-private partnership to support collaboration. Learnings were shared in a summary report prior to a fall 2024 virtual public meeting that gathered public comment from 19 attendees.

A common theme expressed by participants was the need for more time to strategically test and implement the requirements of the rule. In early 2025, the FDA extended the implementation deadline to June 20, 2028.


## Nutrition Communications Network

The Foundation's Nutrition Communications Network amplifies consistent, science-based nutrition information by sharing social media messages, blog posts, and infographics to help consumers take charge of their health — especially those who are most susceptible to chronic diseases. Patient advocacy groups, dietitians, manufacturers, and retailers regularly utilized our guides in 2024. The program was piloted with a focus on the Nutrition Facts label but has expanded to include information on the Dietary Guidelines for Americans and other trusted resources.

## Produce Safety Stakeholder Dialogue

Ensuring the safety of fruits and vegetables calls for novel strategies and a collaborative framework. The Foundation initiated a broad dialogue in 2024, starting with a widely distributed questionnaire that sparked a robust response from across agriculture, industry, academia, and government. The results helped us form working groups that, in 2025, will further articulate perspectives and explore new strategies for produce safety and the development of a collaborative entity to drive progress in key areas, including:

- ✓ Buyer-supplier collaboration for produce safety
- ✓ Produce safety research needs
- ✓ Extension and training
- ✓ One Health/agricultural ecosystem

As the work progresses, the Foundation will release a roadmap based on its findings.

## Cosmetic Labeling Project

How do consumers select cosmetics and related products such as shampoo, deodorant, and skin/shaving care — and do they have the information they need? In 2024, the foundation continued to research the influence of product claims, labeling, and health concerns on consumer choices about cosmetics. The project, a collaboration with the FDA's Office of Cosmetics and Colors, is also exploring consumer understanding of the Agency's regulatory role in cosmetics.

*This project was supported through a cooperative agreement with the Food and Drug Administration.*



# Health Data

## Improving Access to Publicly Available FDA Information


From weather alerts to the latest headlines, Americans expect instant access to news and vital updates. They want that same speed and accuracy when it comes to health and medical information. The FDA's trove of critical health data holds great potential for enhancing public understanding and health care innovation. However, accessing and using the data can be tough for both consumers and professionals.

Issued in July 2024, this comprehensive Foundation report examines how audiences engage with FDA data and identifies challenges and opportunities for making information more transparent and accessible. Our investigation relied on various methods, including a landscape analysis, a review of FDA data sources, a consumer survey, and stakeholder interviews and roundtables. Patients and consumers want easy access to accurate, understandable information. For professional researchers, health care professionals, and intermediary partners, the priority is datasets that are searchable, complete, and easy to integrate into digital tools, such as websites and apps. In addition to presenting the research findings, the report extensively catalogs FDA datasets and includes metadata to improve usability.

Enhancing the transparency and usability of FDA data in innovative ways will expand the benefits of the information, empowering consumers and propelling health research and interventions.

*This project was supported by Arnold Ventures and Lyda Hill Philanthropies.*

> "People usually already have their prescription form or their pharmacy bottle when they go to Google. There's a reasonable chance they end up on the FDA's website."
>
> Website publisher

**Information that Users Seek Online**

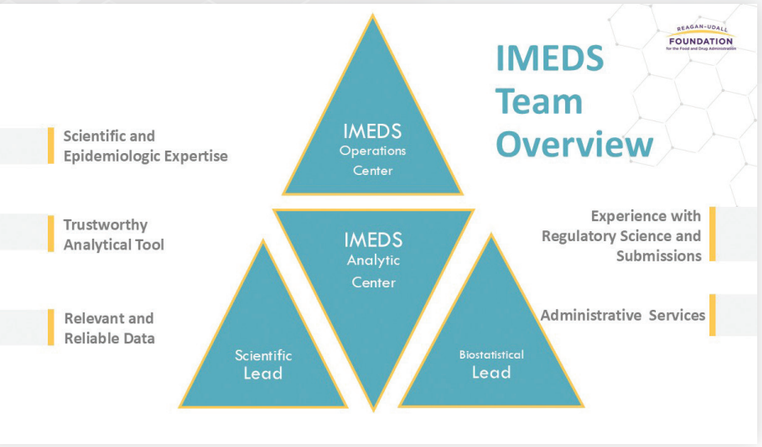
| Patients and Consumers | Health Care Professionals | Professional Researchers | Intermediary Partners |
|---|---|---|---|
| • Health condition or disease<br>• Treatment options<br>• Side effects | • Prescription and over-the-counter drug information, including dosing, treatment duration, and potential side effects | • Pharmacovigilance and other product-specific data<br>• Data for improving treatment and patient care at the individual level<br>• Data for policy considerations<br>• Commercial: real-world data for label expansion and market surveillance | • Data for B2C and B2B digital products<br>• Health care-focused information<br>• Structured data in the form of APIs<br>• Support from the data providers (FDA) |

# Research

Our growing research portfolio advances post-market safety evaluation, innovative tools, and the use of real-world evidence.

## Innovation in Medical Evidence and Development Surveillance (IMEDS)

IMEDS is the Foundation's flagship program for generating real-world evidence to explore the safety of medical products after approval. We leverage trusted FDA-developed "Sentinel" tools to conduct large, rigorous post-market studies. Our record of success brings value to patients, health care professionals, and sponsoring life sciences companies.




In 2024, we led ongoing post-market approval studies of ertugliflozin in patients with type 2 diabetes (Merck) and risankizumab in pregnant women with psoriasis and with Crohn's disease (AbbVie). Deliverables were presented for review by the European Medicines Agency and, in some cases, jointly by the FDA. In October, we held a public webinar to provide an overview of IMEDS.

### Benefits of Working with IMEDS

- Extensive network of more than 75 million patients per year with at least one day of health insurance coverage since 2014
- Streamlined, privacy-protected real-world data harmonized to the Sentinel Common Data Model and analyzed with Sentinel tools
- Deep experience in multi-year post-market studies, from feasibility assessment through final report
- Single point of contact managing the project according to sponsor criteria and timeline

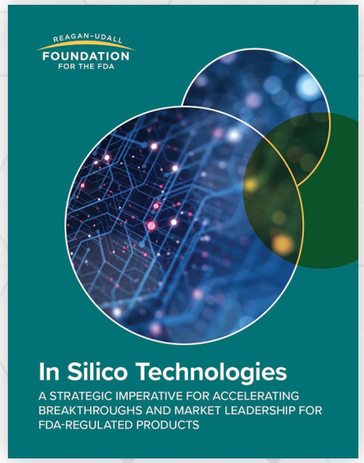
*IMEDS studies were funded by AbbVie and Merck to meet their post-market requirements to the Food and Drug Administration and/or the European Medicines Agency.*

**Advancing Regulatory Science**
Connected science and business to accelerate technology breakthroughs and conduct post-market research

**Disseminating Information**
Published 5 expert reports, journal articles, and frameworks based on key research findings

**Facilitating Engagement**
Drew more than 2,000 participants in public meetings and webinars on scientific research






## Regulatory Science Accelerator: In Silico Technologies

The Foundation continued to build on our Regulatory Science Accelerator with the FDA's Office of Regulatory Science and Innovation to share information about emerging technologies that could revolutionize product development and evaluation. In silico approaches, such as computational modeling and simulation, hold particular promise.

In 2024, Accelerator participants made the business case for in silico investments, expanding on the efforts of a workgroup that had identified alternatives to animal models. Corporations that adopt and leverage these key technologies can bring medical breakthroughs to market rapidly and efficiently, using simulations that reliably predict side effects and real-world outcomes. Generating evidence in new ways may strengthen safety and effectiveness for the benefit of patients while reducing research and development costs.

Our latest publication rounds out a trio of in silico reports available on the Foundation's website.

*This project was supported through a cooperative agreement with the Food and Drug Administration.*

### MYTHS OF IN SILICO TECHNOLOGY ADOPTION

- Business Impact
  - Return on Investment (ROI) not high enough
  - ROI takes too long to realize
- Regulatory considerations
- Modeling is not relevant or applicable across the product lifecycle
- Legacy/industry inertia
- "I'll make revenue with AI; I don't need in silico technologies"
- Lack of accurate input data (e.g., for tissue material properties)
- In silico technologies lack relevance to clinical conditions
- Validation is cumbersome

### OPPORTUNITIES

- Accelerate time to market
- Improve product safety and efficacy profile
- Reduce animal experimentation
- Reduce the number of human subjects in clinical trials
- Greater capacity to explore design space
- Greater potential for scalability
- Transition from in silico models for optimization to in silico models for evidence
- Model reuse with minor tweaks for a similar product profile

# Real-World Evidence

## Real-World Evidence Webinar Series

Since 2021, the Foundation has held public webinars with the FDA on the use of real-world evidence in regulatory decision-making about drugs and biologics. The series previews draft FDA guidance and facilitates feedback.

The first webinar of 2024 shared considerations for researchers planning non-interventional or observational studies to demonstrate safety or effectiveness. The second explored ways to modernize randomized controlled trials by integrating them into local clinical practice. This streamlined model could broaden participation to reach more patients, such as those who live in rural areas or have rare diseases.

*This project was supported through a cooperative agreement with the Food and Drug Administration.*

## Real-World Evidence Consortium for Sickle Cell Disease

The Foundation joined with the American Society of Hematology Research Collaborative (ASH RC) to establish the Real-World Evidence Consortium for Sickle Cell Disease (SCD). The initiative will identify which codes and contexts in electronic health records indicate clinical characteristics of SCD to understand and improve outcomes for the 100,000 Americans living with this serious disease, the nation's most common inherited red blood cell disorder.

The ASH RC and Foundation collaboration represents the intersection of clinical research, clinical care, regulatory affairs, and industry engagement, which uniquely positions these organizations to drive change through the multi-year project. Collaborators will include patients, clinicians, researchers, industry representatives, federal regulators, and informaticians.

*This project was supported by the American Society of Hematology.*

## Oncology Quality Characterization and Assessment of Real-World Data (QCARD) Initiative

The structured, transparent approach of the Oncology QCARD was published in the journal *Pharmacoepidemiology and Drug Safety* in October 2024. A collaboration between the Foundation and the FDA Oncology Center of Excellence, the initiative aims to improve the quality of early study proposals. It identifies core real-world data elements needed by scientific reviewers to evaluate whether proposed data sources are fit-for-purpose and to provide useful feedback to sponsors.

### KEY POINTS

- The Oncology QCARD Initiative developed a core set of standardized study design and data elements to facilitate initial feedback.
- The initial protocol characterization template focuses on clear communication of the following high-level data and study design domains: overall study characteristics, data temporality, population, medical product exposure, comparators, covariates, endpoints, statistical analysis, and data quality assurance plans.
- As a structured, transparent approach to facilitate early review, the template provides an opportunity for enhanced communication with scientific reviewers.

*This project was supported through a cooperative agreement with the Food and Drug Administration.*

## Algorithm CErtaInty Tool (ACE-IT)

ACE-IT is an exciting framework for determining the fitness of an algorithm as an endpoint in a safety study for targeted use cases. With the University of Massachusetts, we developed the original tool to focus on major adverse cardiovascular events. In 2024, we began adapting it to apply to maternal and birth outcomes in pregnancy studies.

*This project was supported by AbbVie, Eli Lilly and Company, and UCB.*


WEBINAR, CLOCKWISE FROM UPPER LEFT: Carla Rodriguez-Watson, PhD, MPH, Reagan-Udall Foundation for the FDA; Lenel James, MBA, FHL7, Blue Cross Blue Shield Association; Darryl Sleep, MD, Amgen; Allen Hsiao, MD, FAAP, FAMIA, Yale New Haven Health System; Anjum Khurshid, MD, PhD, Harvard Pilgrim Health Care Institute

## RAISE

Incomplete and inconsistent data collection in health care settings poses a barrier to the development of safe and effective treatments for everyone. The Foundation and the FDA developed the Real-World Accelerator to Improve the Standard of collection and curation of race and Ethnicity data (RAISE), recognizing that person-centered data can yield more robust, generalizable research results that can facilitate solutions to address unmet clinical need.

In mid-2024, the Foundation released the RAISE Action Framework, a set of strategies and priorities to help leaders in health care systems integrate these data into core operations, allowing administrators, providers, researchers, and insurers to make informed decisions at all levels. A public webinar exploring how organizations can implement the Action Framework to advance health care and health outcomes is available on the Foundation website.

*This project was supported through a cooperative agreement with the Food and Drug Administration.*

## Biosimilars

### Biosimilar Roundtable Series

In collaboration with the FDA's Center for Drug Evaluation and Research, the Foundation led a series of roundtables from August to October 2024 with developers of biosimilars, which offer patients access to more options by mirroring approved biologic drugs.

Participants, drawn from developers with less experience interacting with the FDA, discussed the challenges they face and suggested topics that would be helpful for the FDA to synthesize based on its experience with biosimilars. Their perspectives will inform next steps for the FDA's Regulatory Science Pilot Program, which aims to advance efficient development and approval as part of the Biosimilar User Fee Act. A full report from the series was published on the Foundation's website.

*This project was supported through a cooperative agreement with the Food and Drug Administration.*

TOP CHALLENGES

- Lack of clarity on critical quality attributes (CQAs), acceptable variability, and essential analytical methods
- Availability and variability of reference product lots
- Limited access to comprehensive and transparent data on reference products
- High rates of dropouts due to long follow-up times in patient sampling in pharmacokinetic (PK) similarity studies
- Integrating immunogenicity into PK studies
- Lack of guidance on method selection and CQAs for emerging drug classes like antibody-drug conjugates

### The Science of Immunity

How do you make learning about the immune system fun for middle schoolers while sparking curiosity in the next generation of scientists? By teaching the *Science of Immunity*! This free curriculum is filled with ideas for creative, hands-on activities to challenge students to think critically about science — and what role they might play in it. We developed the curriculum, interactive activities, videos, and curriculum delivery platform with teachers, RTI International, and Dr. Raven the Science Maven.

*This project was supported by the Burroughs Wellcome Fund and the Gordon and Betty Moore Foundation.*



# Controlled Substances

The Foundation drives critical collaborations focused on understanding the evolving overdose crisis, advancing research, and addressing treatment gaps.

### Online Controlled Substances Summit


About 1 in 4 illegal online pharmacies offer controlled substances, exposing consumers to risks of ineffective counterfeit products possibly contaminated with deadly substances such as fentanyl. The Foundation collaborated with the FDA to convene federal leaders along with technology and substance use experts to address this crucial public health issue in the fifth annual Online Controlled Substances Summit in July 2024.

FDA Commissioner Robert M. Califf opened the Summit by describing the growing threat of unregulated prescription drugs sold through websites and social media. Speakers shared perspectives on risks, motivations, and challenges highlighting the need to deliver clear, evidence-based messages using trusted channels to support informed decisions. Part two of the Summit was a smaller meeting with social media, internet, technology, and controlled substance experts working collaboratively to identify immediately implementable actions and long-term strategies aimed at disrupting the trajectory of the current crisis.

The Summit concluded with a call to action to do more than simply warn the public. Preventing overdoses and saving lives requires an innovative, collective response that includes government, academia, advocacy, and technology sectors.

*The projects in the Controlled Substances portfolio were supported through a cooperative agreement with the Food and Drug Administration.*

> "We all live in a shared digital risk ecosystem, which includes social media platforms, e-commerce platforms, internet index websites, communication platforms and, of course, the dark web."
>
> Tim Mackey, MAS, PhD, University of California, San Diego

**Advancing Regulatory Science**

Sought solutions to complex challenges and examined emerging research on potential therapeutics

**Disseminating Information**

Issued 6 impactful reports from public meetings and workgroups

**Facilitating Engagement**

Drew nearly 4,000 participants in roundtables and public meetings, and collected more than 300 public comments on advancing PTSD treatments


FROM LEFT: Marta Sokolowska, PhD, FDA; Paula P. Schnurr, PhD, Department of Veterans Affairs; Neeraj "Jim" Gandotra, MD, Substance Abuse and Mental Health Services Administration; Leith J. States, MD, MPH, MBA, FACPM, Office of the Assistant Secretary for Health, Department of Health and Human Services; Miriam J. Smyth, PhD, Department of Veterans Affairs; Bernard Fischer, MD, FDA

### Advancing Treatments for Post-Traumatic Stress Disorder

The Foundation convened a hybrid public meeting on PTSD treatment needs to support the FDA's commitment to listening to, and engaging with, patients. Agency representatives and officials from the Departments of Defense, Health and Human Services, and Veterans Affairs and the Substance Abuse and Mental Health Services Administration explored cross-agency initiatives to propel the development and approval of therapeutics, including psychedelics.

Public comments included voices of veterans and others affected by PTSD, as well as families, advocates, researchers, and drug developers. Concerns about high rates of suicide infused the comments with a sense of urgency and frustration. Commenters observed that current options, including two approved drugs and various behavioral health treatments, do not work for everyone and are not always accessible.

Participants called for continued investment, creativity, and continuous learning in collaboration with patients and the public and private sectors.

> "This is not just a crisis. It's an emergency that demands national attention."
>
> PTSD patient advocate

### Psychedelic Clinical Study Design

Psychedelics and their potential therapeutic uses were the subject of a two-day public meeting. Speakers reviewed the FDA's first draft guidance on psychedelic research and development for the treatment of medical conditions. Despite scientific and regulatory advancements, many questions remain. Continued progress will require broad collaboration and rigorous studies that optimize the interpretability of results.

### Understanding Current Use of Ketamine for Emerging Areas of Interest

This hybrid public workshop, in collaboration with the FDA, focused on ketamine: current and emerging clinical uses, evidence gaps, and safety concerns. Participants learned about the drug's history and its shifting use as an approved anesthetic agent and an off-label treatment for chronic pain and psychiatric disorders, including depression. Participants weighed policy and regulatory opportunities and challenges, including as they relate to online sales.

### Mitigating Risks from Human Xylazine Exposure

In February, the Foundation released a report on our 2023 public meeting on "Mitigating Risks from Human Xylazine Exposure." Xylazine is an approved veterinary drug used as a dangerous cutting agent in illegal drugs. Concrete mitigation strategies include leveraging and improving real-time surveillance and rapid response, data collection and information sharing, and person-centered care. The final takeaway was that the opioid crisis is bigger than one substance and demands a comprehensive response.

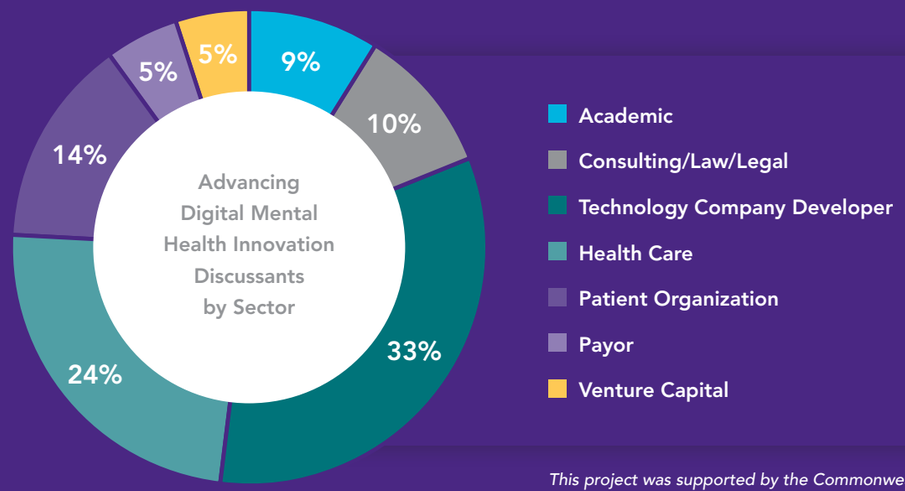


## ADVANCING DIGITAL MENTAL HEALTH INNOVATION

More than 1 in 5 Americans need mental health care. Digital tools could help improve access and affordability, but their proliferation has outpaced the ability of developers and regulators to adapt.

The Foundation convened a conversation among experts from across the mental health ecosystem — patients, providers, and the technology sector — to probe barriers to innovating and regulating effective interventions in this complex field. They identified opportunities for greater communication, collaboration, and flexibility that could improve consumer options and outcomes.


| CHALLENGES | SOLUTIONS |
|---|---|
| Health care professionals, patients, and users lack information to make informed care decisions about using digital tools | FDA and NIH should continue leading collaborative efforts to expand clinical measures and best practices in clinical trial design for digital mental health tools |
| Mental and behavioral health industry includes new entrants to federal regulatory oversight | FDA should strengthen its proactive approach to educating digital health product developers |
| Review for FDA marketing authorization and payor reimbursement are conflated | Non-government organizations could play a bigger role in supporting mental and behavioral health stakeholders |
| Rapidly evolving landscape requires regulatory flexibility | FDA should consider enabling greater flexibility in mental and behavioral health submissions |
| Distinct efforts spanning multiple federal agencies lead to perceived inefficiencies | Government agencies should enhance collaboration through continued interagency task force efforts for digital mental health tools |




*This project was supported by the Commonwealth Fund and through a cooperative agreement with the Food and Drug Administration.*

# Patient Listening Sessions

The Foundation helps the FDA connect with patient communities and understand what matters most to them. Through these vital engagements, participants share their health experiences and needs, providing the FDA with insights to inform its decision-making. In 2024, we collaboratively organized 14 patient listening sessions on a range of topics — most at the request of patient groups.

| |
|---|
| **Attention-Deficit/Hyperactivity Disorder (ADHD)*** |
| **Adult-Onset Leukoencephalopathy with Axonal Spheroids and Pigmented Glia (ALSP)** |
| **Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)** |
| **Congenital Cytomegalovirus Infection (cCMV)** |
| **DLG4-Related Synaptopathy** |
| **Facioscapulohumeral Muscular Dystrophy (FSHD)** |
| **KCNT1-Related Epilepsy** |
| **MEF2C Haploinsufficiency Syndrome (MCHS)** |
| **Myhre Syndrome** |
| **Neurofibromatosis Type 1 (NF1)/Cutaneous Neurofibroma (cNF)** |
| **Okur-Chung Neurodevelopmental Syndrome (OCNDS)** |
| **Post-Traumatic Stress Disorder (PTSD)** |

**Three sessions*

# Fellowship in Regulatory Science and Innovation

The Foundation's fellowship program provides opportunities for growth and mentorship to professional school graduates while supporting the FDA's efforts to expand the regulatory science workforce. In partnership with Howard University, we selected Perpetue Backer, PhD, as the Foundation's 2024–25 Regulatory Science Fellow. Fellows rotate through key functions at the Foundation, building their research, communication, engagement, and operations experience as well as gaining insight into the FDA's regulatory role and processes.

*This project was supported by BeiGene, Burroughs Wellcome Fund, PhRMA, and Roche Diagnostics.*

> "I engaged with a variety of stakeholders and learned to synthesize their insights into actionable recommendations that shape research priorities and improve health outcomes."
>
> Perpetue Backer, PhD, 2024–25 Fellow

# 2024 Innovations in Regulatory Science Awards

The Foundation's annual Awards recognize achievement in improving scientific knowledge, advancing scientific process, and expanding the application of science to improve public health. Leaders and visionaries came together on December 10, 2024, to celebrate the awardees' exceptional contributions to the future of regulatory science.

Rachele Hendricks-Sturrup, DHSc, MSc, MA, accepting the Innovation award

## INNOVATION AWARD

**Real-World Evidence Collaborative**

Accepted by **Rachele Hendricks-Sturrup**, DHSc, MSc, MA, Research Director, Real-World Evidence, Duke-Margolis Institute for Health Policy

The Real-World Evidence Collaborative spearheaded innovation in regulatory science by advancing the application of real-world evidence. Its International Harmonization of Real-World Evidence Standards Dashboard, launched in 2023, has already made important strides toward fostering worldwide alignment on data and evidence standards.

Steven A. Grossman, JD, accepting the Advocacy/ Policy award

## ADVOCACY/POLICY AWARD

**Steven A. Grossman**, JD, Co-Founder and Executive Director, Alliance for a Stronger FDA

Steven Grossman has demonstrated his unwavering commitment to public health through a career dedicated to public service and patient and research advocacy. Thanks in part to the Alliance for a Stronger FDA, a broad coalition he co-founded in 2006, the Agency's appropriated budget more than doubled.

Jeff Shuren, MD, JD, accepting the Leadership award

## LEADERSHIP AWARD

**Jeff Shuren**, MD, JD, Center Director Emeritus (Retired), FDA

During his distinguished leadership of the FDA's Center for Devices and Radiological Health, Dr. Shuren presided over a remarkable expansion of patient access to safe, innovative medical devices. He played a key role in critical initiatives, including the Breakthrough Devices Program, Patient Engagement Program, Digital Health Center of Excellence, and National Evaluation System for Health Technology.



1

2

3

1. Then-FDA Commissioner Robert M. Califf, MD, MACC, participates in an on-stage conversation with Foundation Board member and former Commissioner Andrew C. Von Eschenbach, MD, during the 2024 Innovations in Regulatory Science Awards.
2. Susan C. Winckler, RPh, Esq., Foundation CEO, and Richard L. Schilsky, MD, FASCO, Foundation Board Chair
3. FROM LEFT: Richard Schilsky, MD, FASCO; Jeff Shuren, MD, JD; Rachele Hendricks-Sturrup, DHSc, MSc, MA; Steven A. Grossman, JD; Susan C. Winckler, RPh, Esq.

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**Advancing Regulatory Science**

Honored and inspired innovation, advocacy, and leadership

**Facilitating Engagement**

Welcomed more than 300 participants from consumer advocacy, public health, research, industry, and government


# 2024 Annual Public Meeting

The Foundation convened the annual public meeting of its Board of Directors on May 7, 2024, joined by FDA leaders and engaged attendees.

Board Chair Dr. Richard L. Schilsky highlighted the Foundation's efforts to inform FDA innovation, including an enhanced focus on patient and consumer-centered activities as reflected in our strategic framework. He also shared wide-ranging progress toward improving precision medicine, evidence generation, and the communication of science to the public.

A town hall-style discussion moderated by Foundation CEO Susan C. Winckler sparked insights from a panel of FDA leaders: Center for Biologics Evaluation and Research Director Dr. Peter Marks, Center for Drug Evaluation and Research Deputy Director Dr. Marta Sokolowska, Deputy Commissioner for Human Foods Jim Jones, Center for Devices and Radiological Health Director Dr. Jeff Shuren, Center for Tobacco Products Deputy Director Michele Mital, Center for Veterinary Medicine Deputy Director Dr. William Flynn, and Regulatory Affairs Deputy Associate Commissioner Douglas Stearn.

Commissioner Robert M. Califf underscored FDA priorities, including tobacco enforcement and rare diseases, before taking questions from the audience.

1. FROM LEFT: Marta Sokolowska, PhD, FDA; Jeff Shuren, MD, JD, FDA; Jim Jones, MA, FDA
2. Richard L. Schilsky, MD, FASCO, Foundation Board Chair
3. Susan C. Winckler, RPh, Esq., Foundation CEO



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*As of 12/31/24*

*The Foundation's authorizing statute establishes requirements for the construct of its Board of Directors, including designated seats for consumers, health professionals, academia, and regulated industry.*

# Financials

| | 2024 | 2023 |
|---|---|---|
| **REVENUE AND SUPPORT** | | |
| FDA Direct Funding* | $1,650,000 | $1,650,000 |
| Government Grants** | 2,047,045 | 2,527,302 |
| Grants and Contributions | 690,143 | 197,363 |
| Contracts | 3,407,304 | 3,399,686 |
| Fundraising | 414,236 | 353,530 |
| Miscellaneous and Interest Income | 179,276 | 204,342 |
| **Total Revenue and Support** | **$8,388,004** | **$8,332,223** |
| **EXPENSES AND CHANGES IN NET ASSETS** | | |
| Program Services | 7,489,729 | 7,348,254 |
| Supporting Services | | |
| Management and General | 254,642 | 253,700 |
| Fundraising | 281,631 | 301,287 |
| Total Supporting Services | 536,273 | 554,987 |
| TOTAL EXPENSES | $8,026,002 | $7,903,241 |
| CHANGE IN NET ASSETS | $362,002 | $428,982 |

**The Reagan-Udall Foundation for the FDA's operations are partially supported by direct funding from the U.S. Food and Drug Administration (21 USC Chapter 9, Subchapter VII, Sec. 379dd(n)).*

***Government grants include support from the U.S. Food and Drug Administration. The work products are those of the grantee and do not necessarily represent the official views of, nor an endorsement by, FDA, HHS, or the U.S. Government. For more information, please visit FDA.gov.*



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## WE VALUE YOUR TRUST

The Reagan-Udall Foundation for the FDA has earned a Four-Star rating from Charity Navigator, the nation's largest independent evaluator of philanthropic organizations. This top designation is awarded to highly effective, trustworthy nonprofits that meet or exceed standards across key performance areas, including financial health and governance. The rating reflects the Foundation's strong commitment to transparency, accountability, efficiency, and responsible stewardship.

Charity Navigator
FOUR-STAR
2025

William N. (Bill) Hait, MD, PhD
Board Member, 2022–25

# Thank you!

As his service on the Foundation's Board of Directors concludes, we extend our sincere appreciation to Dr. Bill Hait for his leadership and commitment to advancing regulatory science and the work of the Foundation.

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*as of 12/31/24*



## STRATEGIC FRAMEWORK

Helping the FDA do more to protect and promote the public's health

### MISSION

Advance the mission of the Food and Drug Administration to modernize product development, accelerate innovation, and enhance product safety.

### VISION

A world where regulation informed by science improves product innovation and public health.

### 3-YEAR OUTCOME

**Progress Toward Our Vision:** The Foundation manages a suite of programs that assist the FDA to engage with external stakeholders and that facilitate evidence generation, improve public understanding of the FDA, and deliver improved health information to the public.

### VALUES

- **Engagement**
- **Innovation**
- **Evidence**
- **Patient and Consumer Centered**

### PILLARS

**Advancing Regulatory Science**

**Supporting Development and Dissemination of Reliable Information**

**Facilitating Engagement and Information Exchange**

### GOALS

Encourage innovation in regulatory science and career development

Improve public understanding of the FDA and the risks and benefits of FDA-regulated products

Support inclusion of all affected populations in research

Facilitate multi-stakeholder collaboration to accelerate evidence generation and dissemination

### STRATEGIES

Provide data assets, evaluation tools, and career development opportunities to help the FDA assess the risks and benefits of regulated products

Identify priority areas for consumer, patient, and provider education in areas of emerging science

Improve provider, patient, and consumer access to regulated products throughout product life cycles

Enable expert analysis, candid discussion, and actionable recommendations on issues relevant to the FDA mission

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