The Role of Standardized Study Data in Efficient and Effective Drug Application Reviews

Lilliam Rosario, PhD

Director of the Office of Computational Science (OCS)

Office of Translational Science (OTS)

Center for Drug Evaluation and Research (CDER)

U.S. Food and Drug Administration (FDA), United States

Impact of Standardized Data

Access to standardized study data has revolutionized regulatory review and enabled FDA to apply increasingly innovative and automated solutions to expedite the review process and empower reviewers with new tools and technologies

Electronic Study Data

Per FD&C Act Section 745A(a), drug application sponsors must use the standards defined in the FDA Data Standards Catalog starting 24 months after the issuance of final guidance for a specific submission type.

- Reviewing study data in a timely manner is critical for FDA's review process (e.g. Reviewers have 30 days to review an IND application)
- When sponsors submit data to the FDA in a reliable and accessible format, it improves efficiency and timeliness of review decisions
- CDISC Standards enable FDA to streamline the review process:
  - Reduce time for reviewers to locate and identify study data
  - Reduce the burden on sponsors and reviewers from Information Requests
  - Reduce review time by enabling the use of commercial off the shelf reviewer's tools to automate review analyses
  - Support data driven decisions by applying data mining and data analytic techniques

Evolution of Study Data Submissions

Paper/PDF Study Data

Electronic Legacy Study Data

Standardized Study Data

Standardized Analysis Data

Manual Review Processes and Techniques

Increased Data Quality, Consistency, Accuracy, Traceability

Automated Review Processes and Techniques

This is where we started…

Paper/PDF Submissions

Difficulty finding data, involved manual searching through paper documents or PDF files

Inefficient exploration of data, which often meant transcribing or copying and pasting into Excel

Time-consuming formatting and manipulation for incorporating safety findings into reviews

Electronic Legacy Study Data

Locating data within a submission became easier

Different conventions across studies and applications limited usefulness

Difficulty combining data and using standardized tools

This is the evolution…

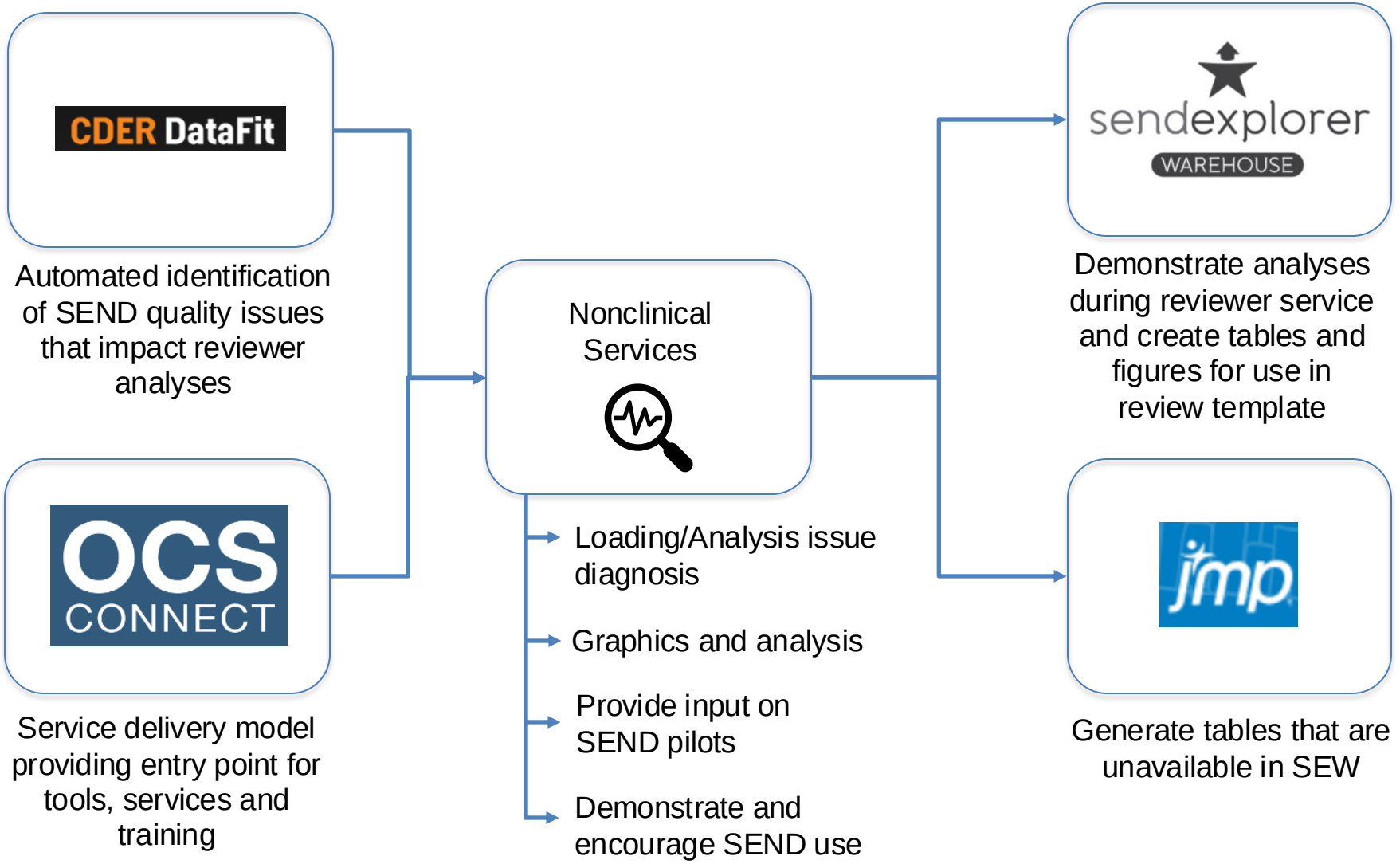
- **Consistent and predictable presentation of data allows for automation**
- **Same structure from sponsor to sponsor and study to study**
- **Consistent general framework for organizing study data**
- **Shared language and common understanding of information collected in studies**

What We Do

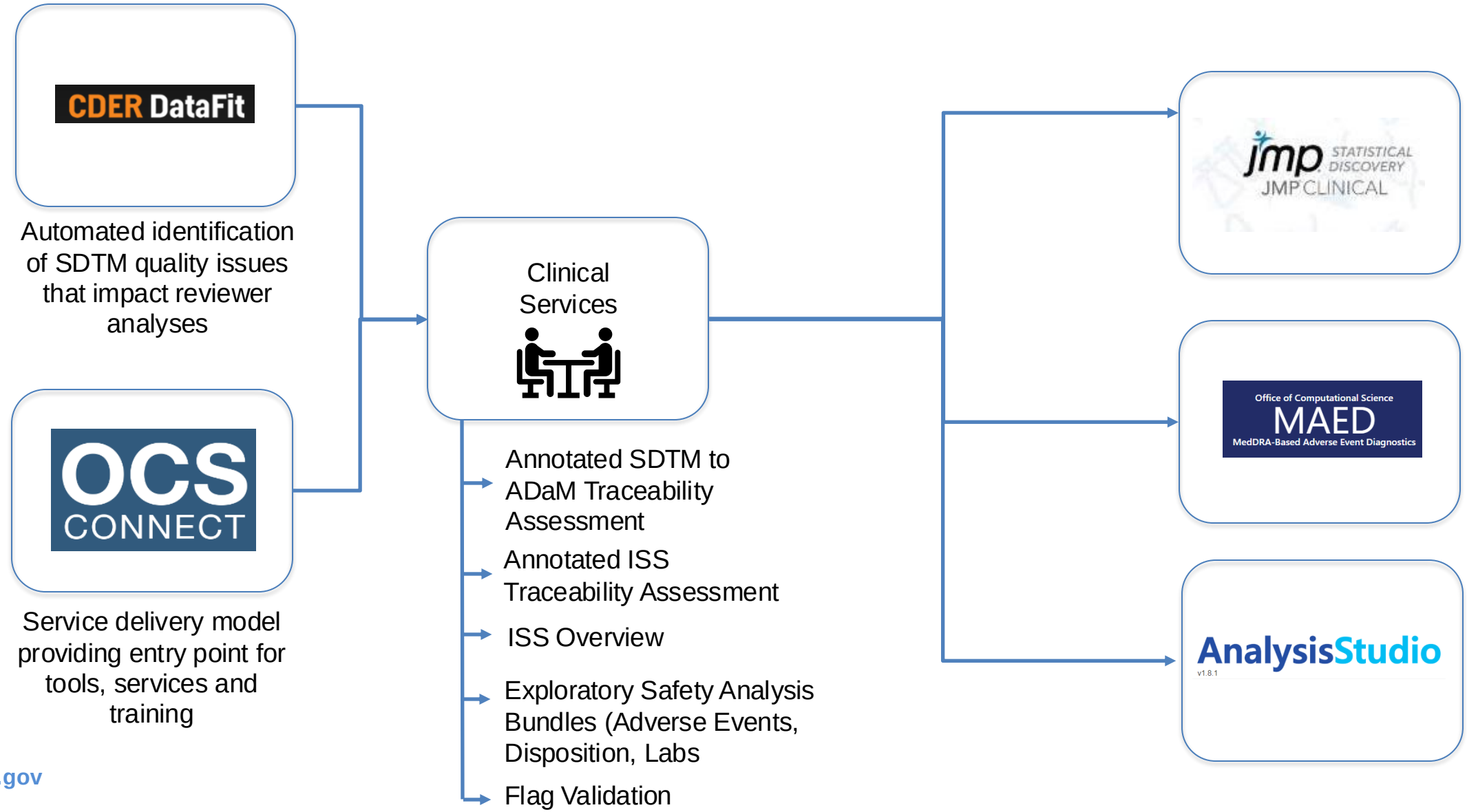
OCS offers a range of services, tools, and support options to meet our customer needs…

…and help them imagine the possibility of something else

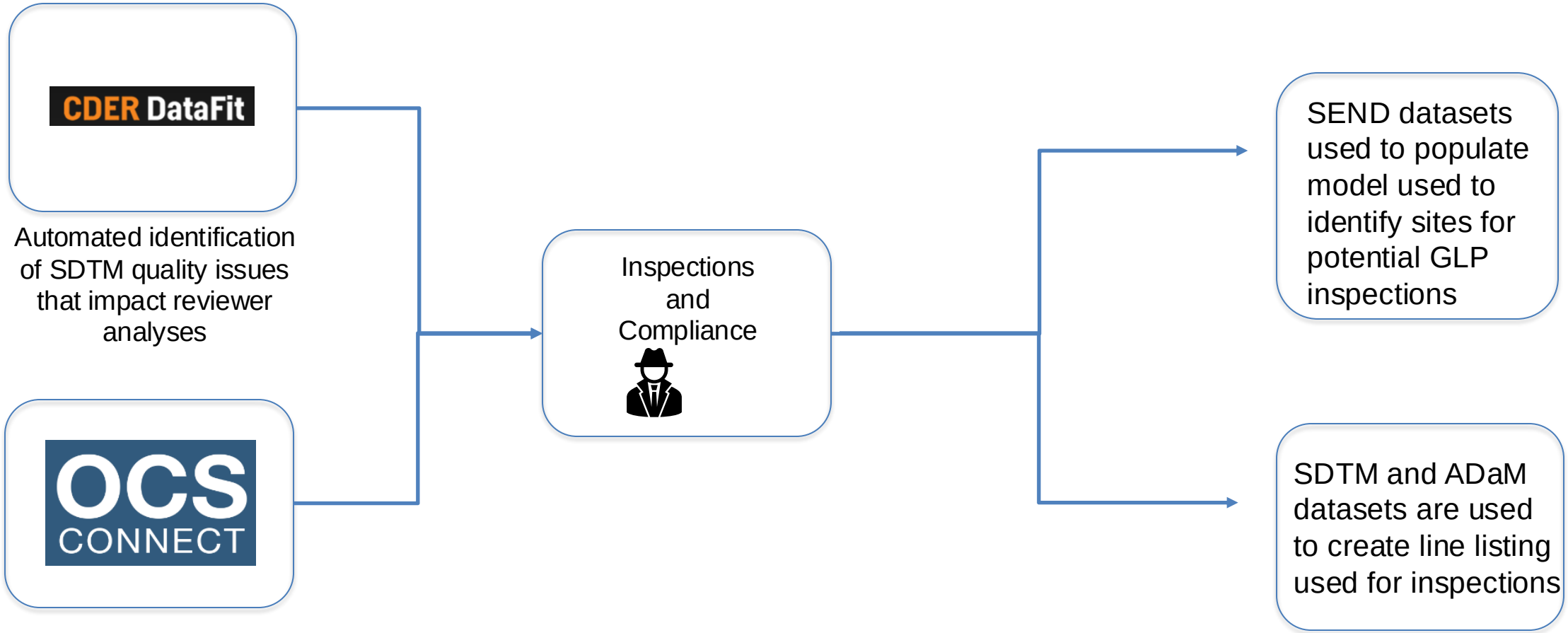
Nonclinical Services Delivery



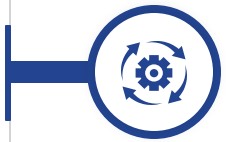
Clinical Services Delivery



Inspection and Compliance

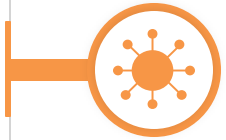


Horizon scanning



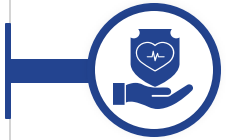
Advances in Clinical Trials

- Introduces remote participation and wearables in decentralized clinical trials, enables predictive analytics to identify potential safety issues and predict patient responses, uses machine learning to facilitate adaptive trials based on interim results, uses EHRs to gather real-world data and streamline patient recruitment



Enhances Effective Regulatory Oversight Pre-Market

- Data analytics, predictive modeling, and real-world evidence generation strengthen ability to monitor product safety, efficacy, and quality. Advanced analytics and AI streamline the review process by analyzing vast amounts of clinical trial data, identifying patterns, and predicting outcomes more accurately.



Real-Time Monitoring Post-Market

- Digital tools enable continuous monitoring of drug performance post-approval, facilitating quicker responses to safety concerns and efficacy issues.

Regulatory Automation

- Automation of routine tasks can improve efficiency, allowing for more resources to focus on complex evaluations.

Protects Public Health

- In an increasingly global healthcare landscape, regulatory agencies must stay ahead of the curve, remain relevant, and deliver value to their stakeholders.

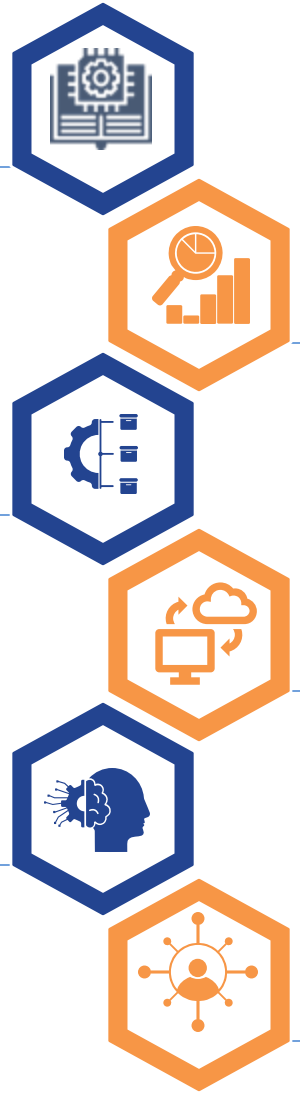
Technological Innovation

- Artificial Intelligence and Medical Products
  - Since 1995 the FDA has received over 300 submissions for drugs and biological products with AI components, and more than 700 submissions for AI-enabled devices.
  - Submissions have included aspects related to drug discovery and repurposing, enhancing clinical trial design elements, dose optimization, endpoint/biomarker assessment, and postmarket surveillance.
  - CDER AI Council meets the requirements set by President Joe Biden's Executive Order (EO) 14110 to promote the safe, secure, and trustworthy development of AI technologies and a follow-up memorandum from the Office of Management and Budget.
- Facilitates FDA's internal operations and regulatory processes to increase productivity, opportunity, and efficiency
- Enables processing and analysis of complex data faster
- Streamlines workflows and automates administrative functions to enable reviewers to focus on complex activities

Technologies for Innovation

Programming languages for statistical computing and graphics

Using statistical computing and graphics to improve efficiency, effectiveness of regulatory review.



Big Data Analytics

Using large and complex data sets to uncover insights and trends that can inform decision-making and drive business growth.

Artificial Intelligence

Using machine learning and other AI technologies to support regulatory review.

Cloud Computing

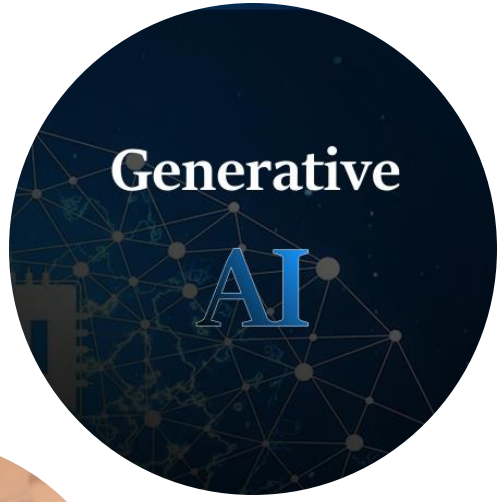
Leveraging the capabilities of cloud-based infrastructure and platforms.

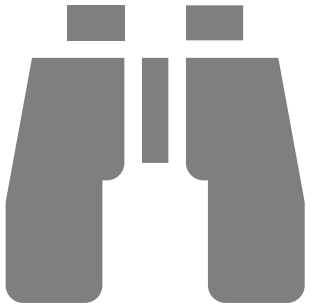
Machine Learning

Using algorithms and statistical models to enable computers to learn from data and make predictions or recommendations.

Robotic Process Automation

Automating repetitive business processes using software robots that mimic human actions.





- New paradigm of drug development and clinical research
- New paradigm in regulatory review
- How do we use technology?
- How do we collaborate?

QUESTIONS?