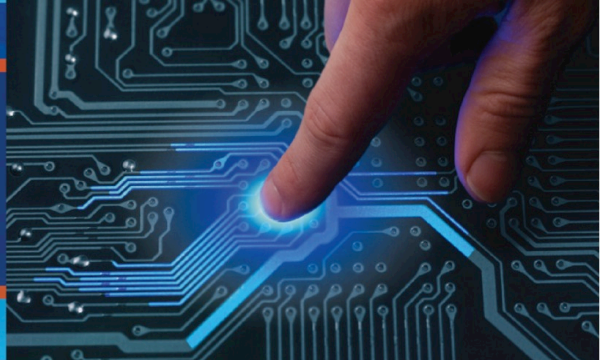




**FDA/PhUSE Annual
Computational Science Symposium**

March 19 - 20th 2012

Silver Spring Civic Center | Silver Spring Maryland, USA



CDER Computational Science Center



Better Data, Better Tools, Better Decisions



Disclaimer

- The views presented here represent those of the speaker and should not be taken to represent guidance or policy on behalf of the Food and Drug Administration



CSC Progress

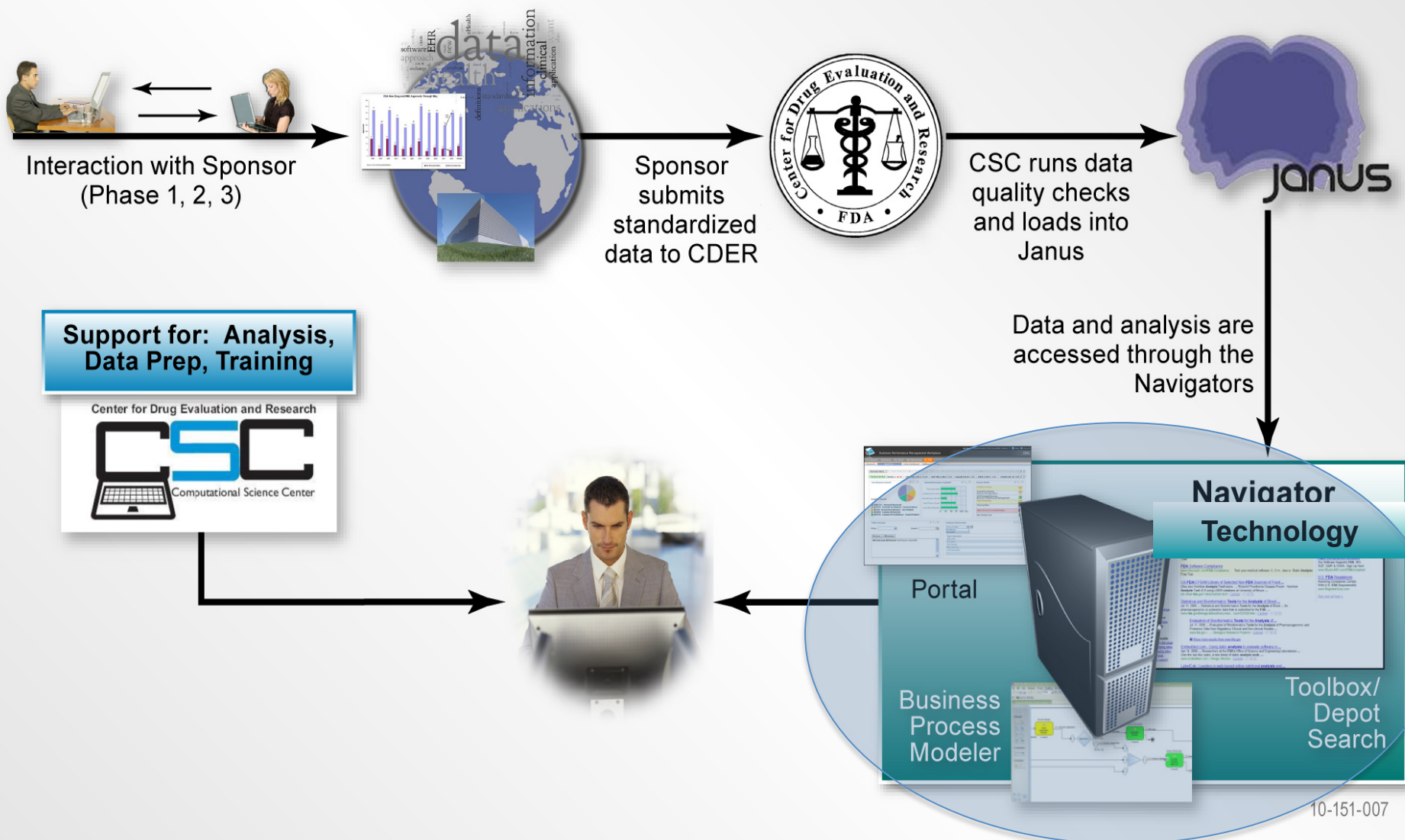
Challenges and Gaps Identified from FDA Initiatives: CDER Perspective

March 19, 2012

Chuck Cooper, M.D.
Computational Science Center, OTS, CDER

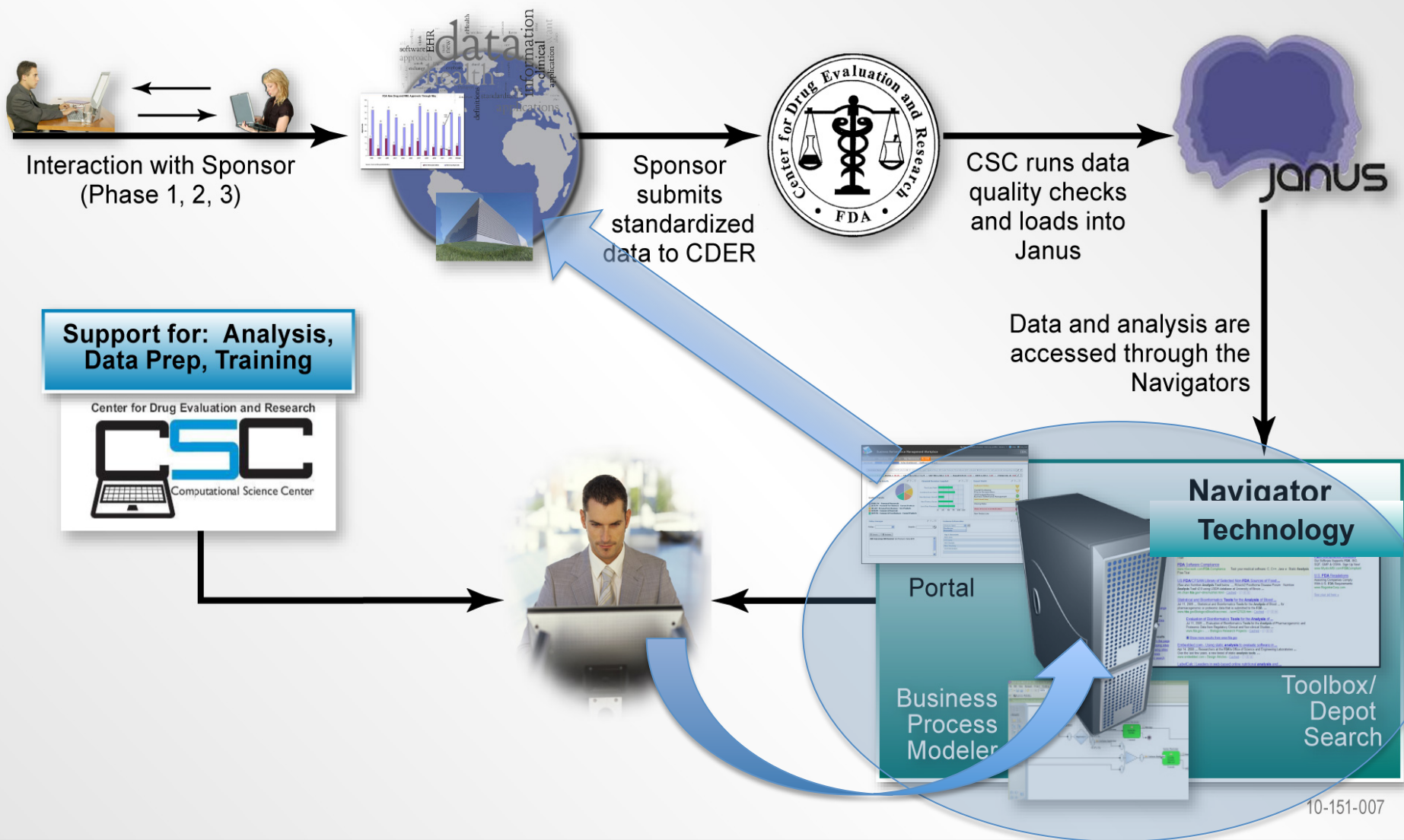


Future State





Future State – Many Challenges





Focus of Work Groups

- The focus of the Work Groups (WGs) represents critical pieces of the puzzle for FDA to build its modern review environment
 - Many things the WGs could work on
 - Focus that has been identified represents critical needs
 - Overlap with industry needs
 - Together, we have the opportunity to identify possible solutions that help us both
- Without PhUSE/FDA WGs, FDA would have proceed with development of solutions alone
 - Less than ideal
 - Solutions have implications to industry
 - Would not leverage broader stakeholder expertise
 - Unique opportunity for collaborative problem solving
- Review of WG focus in relation to FDA development needs

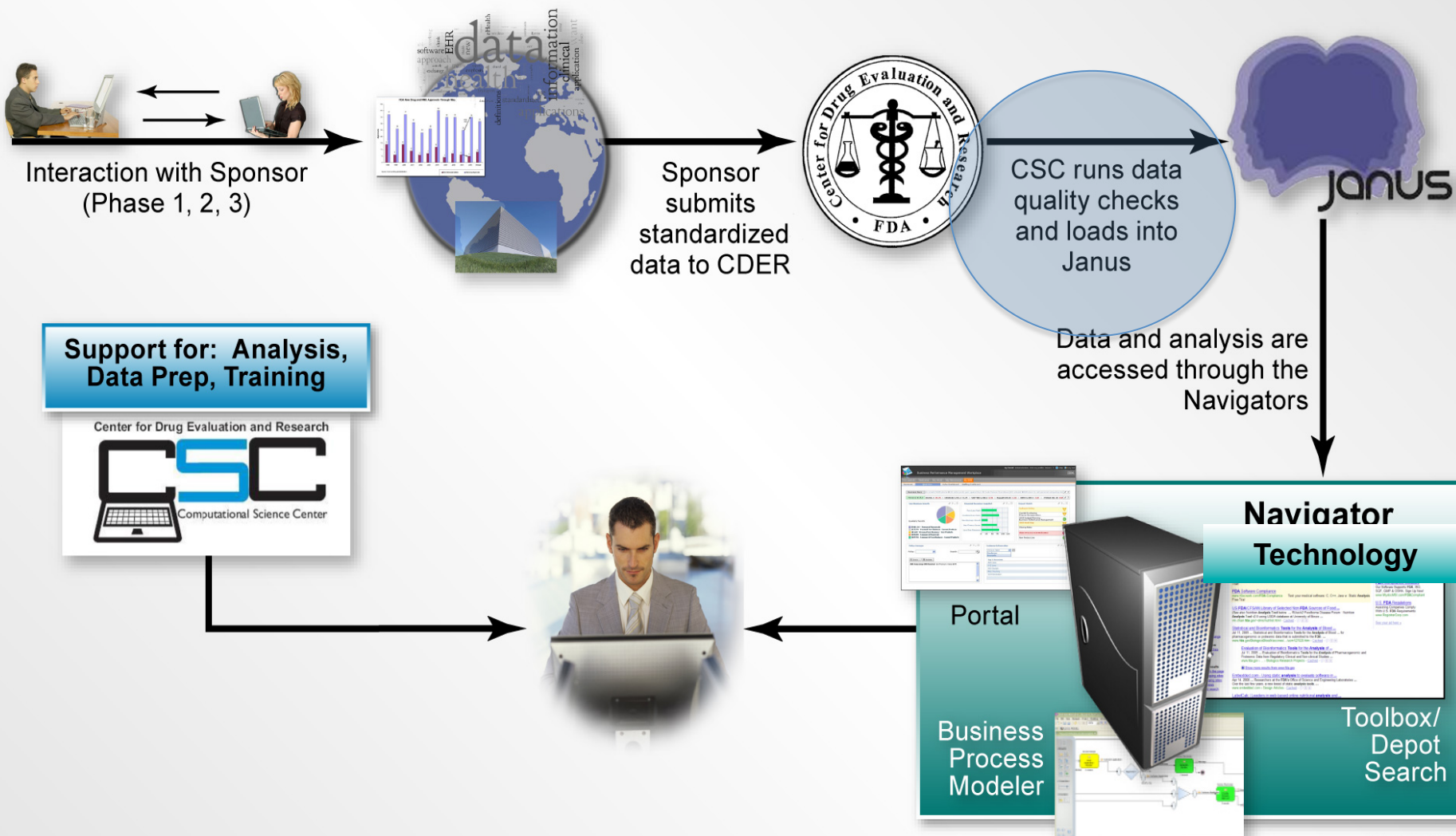


Focus of WG1 – Data Validation

- Unlike any individual company, FDA doesn't control the quality or type of data we receive
- Major impediment to advancing our review environment
- Cannot build review tools that account for non-standard data
- A review environment which provides more advanced review capabilities will have data requirements
- FDA needs to be able to rapidly evaluate data when it hits the door to determine adequacy
- A data validation capability is critical



Work Group 1: Data Validation





Focus of WG1 – Data Validation

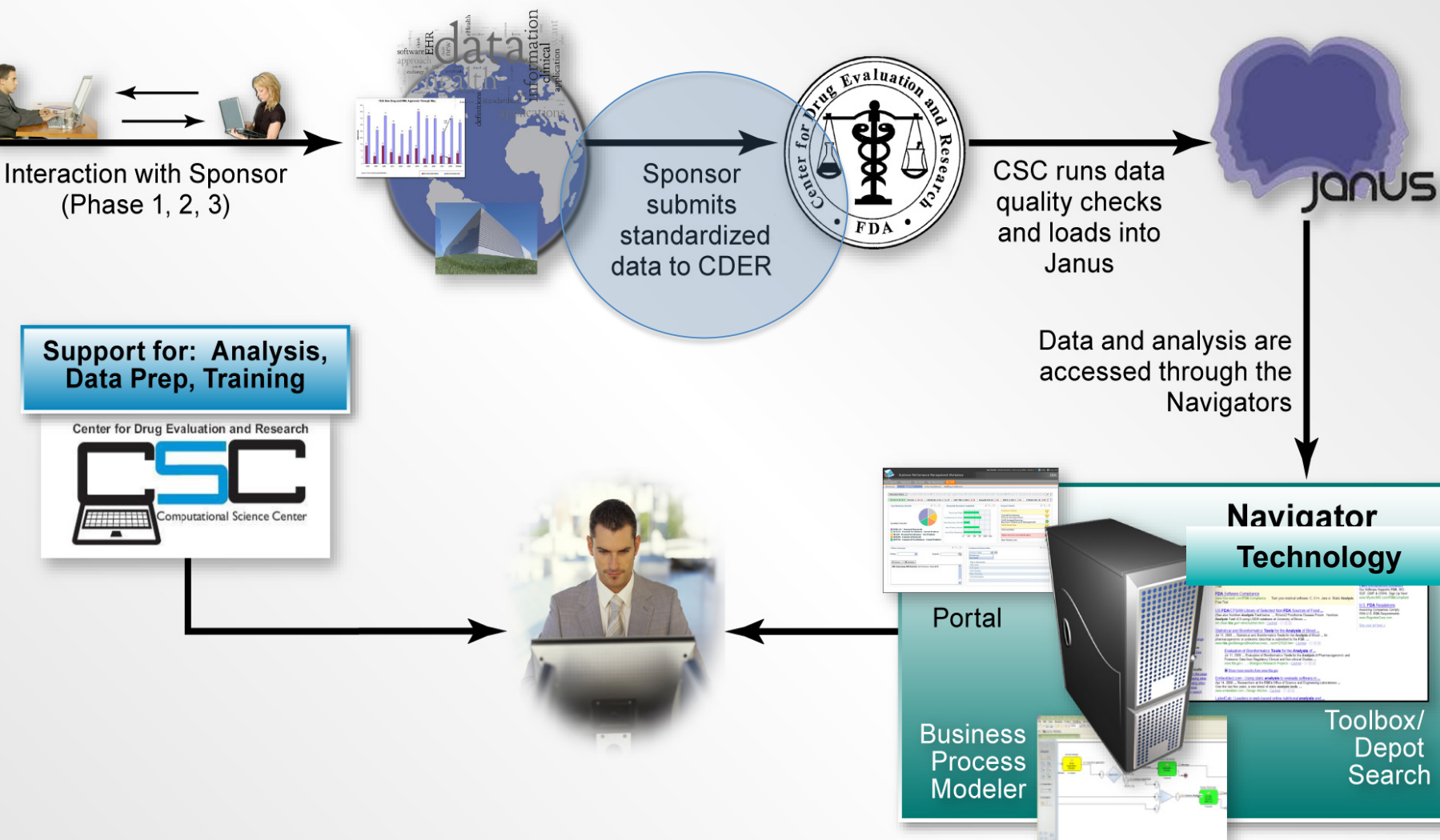
- Obstacles to building an effective data validation capability
 1. No organized way to institute data validation checks
 2. Ineffective/inefficient development process
 3. Testing/repair of existing data checks



Focus of WG1 – Data Validation

1. Assess and improve existing data validation checks
 - goal is to evaluate the effectiveness of existing rules
 - provide better categorization, descriptions, and rationale to assist sponsors in preparation of data for submissions
 - Example: laboratory units
2. Develop guideline documentation for data validation check development
 - focused at the developers of new data standards
 - expertise in understanding how the data will be used
 - avoid deferring the creation of checks to those who are SMEs
3. Develop and support change management process
 - need to organize the development process
 - entering a phase of rapid development
 - Need to be able to approach data validation development and implementation in an organized way

Work Group 2 Data: Standardizing Data for Inspection Site Selection Process





Work Group 2- Standard Data for Site Selection

1. Office of Scientific Investigations has been in a phase of rapid development and innovation
2. Goal is to improve efficiency, effectiveness, and predictability with regard to data quality assessment
3. Advanced review capabilities are under development or have been developed
4. Reviewer needs have been accounted for
5. Data requirements of the tools have been identified

QUESTION: How can we receive data from sponsors which meet the requirements/needs of the reviewers and new review tools?

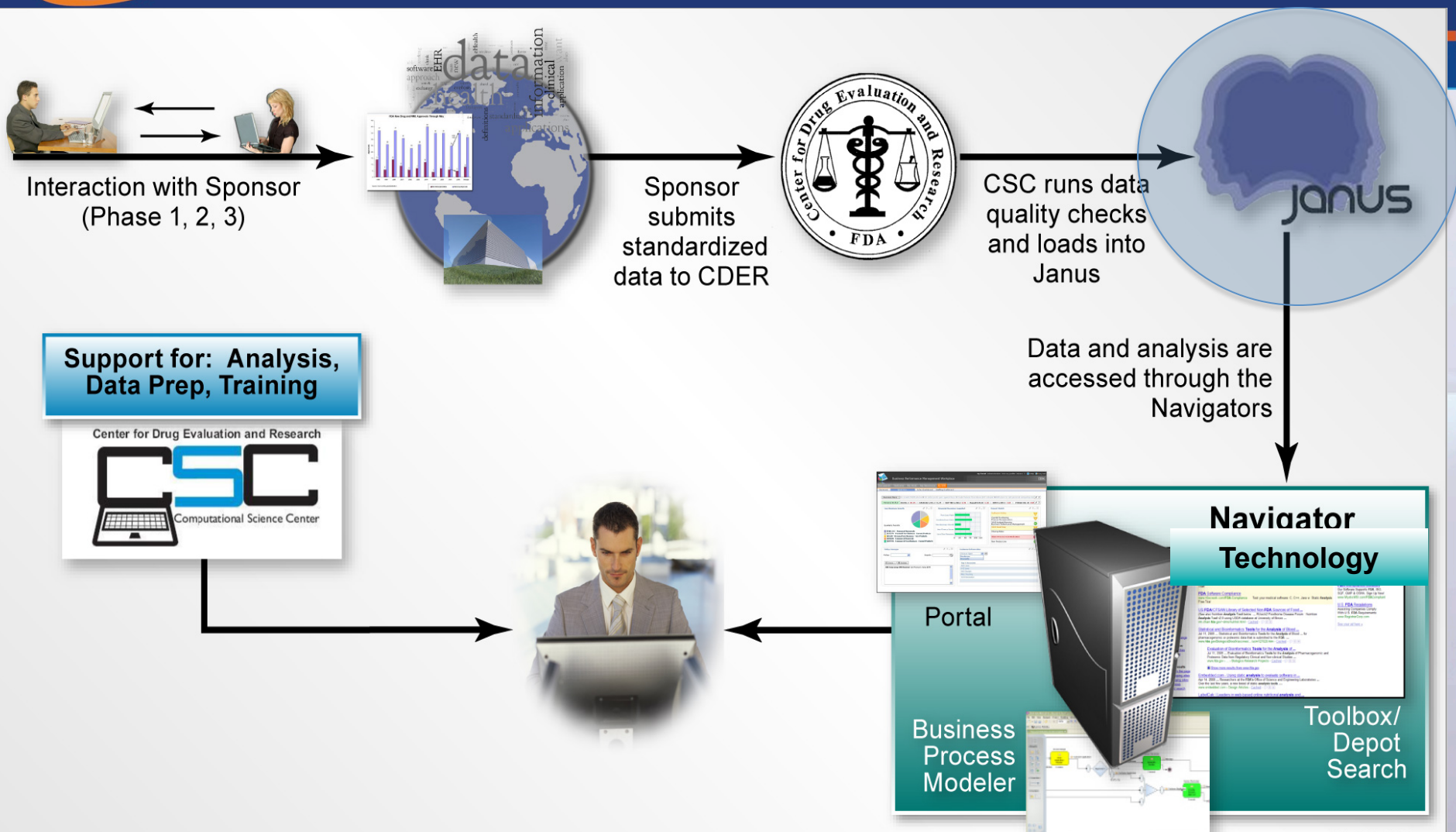


Focus of Work Group 2

1. The specific requirements for the data to be submitted and the relationship to existing data standards (CDISC: SDTM, AdAM)
2. Why the specific data is requested and how it will drive the site-selection process
3. How is the data to be submitted within the context of the NDA and how it will be managed
4. What are the implications for sponsors for the development of risk-based monitoring plan and processes



Work Group 3 – Data Integration





Work Group 3 – Challenges of Integrating and Converting Data across Studies

- Ability to analyze data across studies presents unique requirements
- The concept of a clinical trials repository is entirely reliant on those requirements
- Should be driven by analytic needs and data uses
- Implications on
 - sponsor submission of integrated data sets
 - approach to data conversion activities

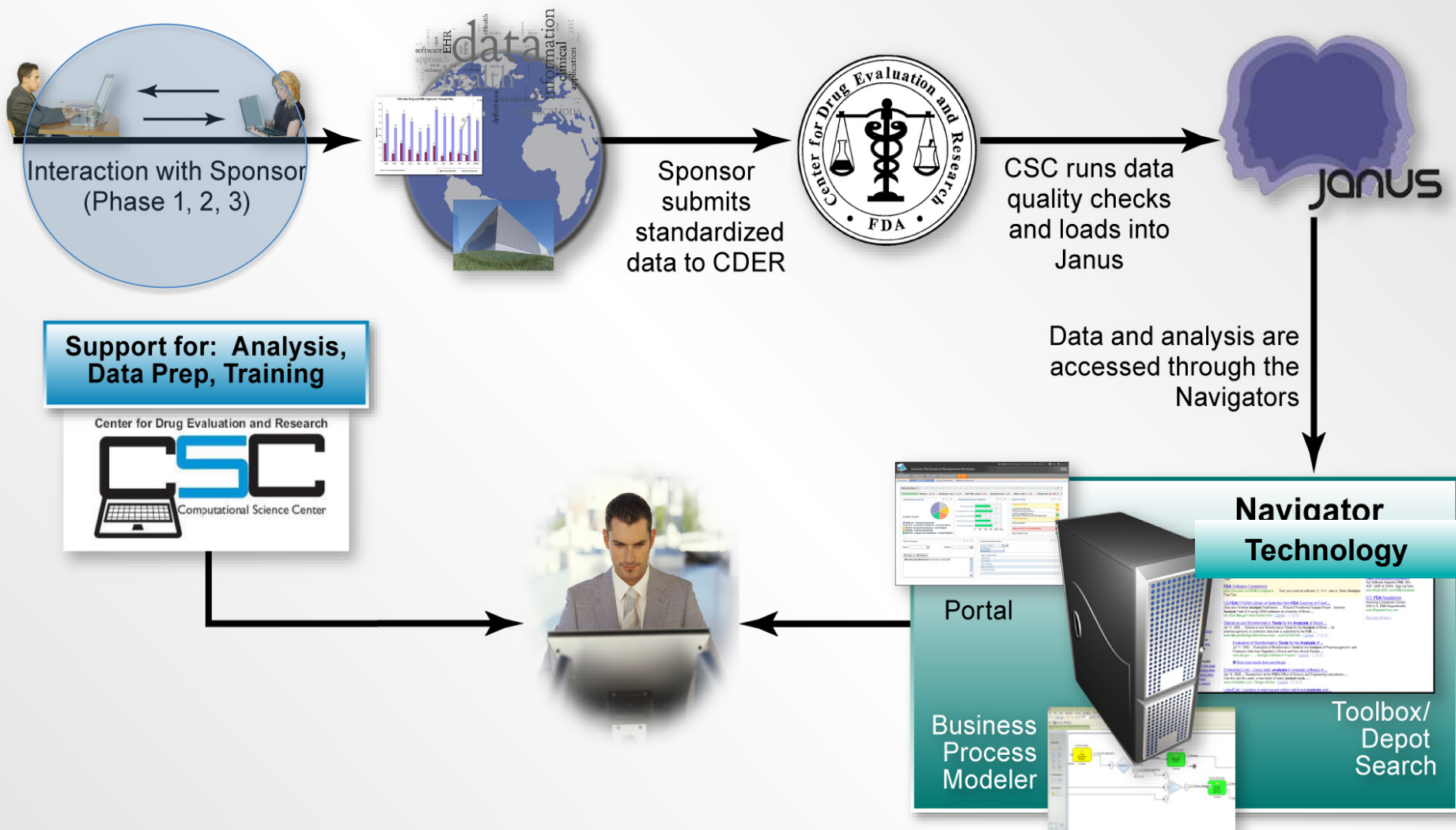


Focus of Work Group 3

- The mechanics of integration
- Tension between Traceability and Integration
- Traceability
 - Ability of analysis data sets to support key analyses in the CSRs
 - Ability to confirm the veracity of the analysis data sets using the SDTM
 - Ability to confirm content of SDTM data sets by referring back to the CRFs
 - The types of integration, use cases
- Impact on data conversion processes



Work Group 4 – Data Standards Implementation Issues





Focus of Work Group 4

- Where is industry struggling with implementation?
 - And where are these struggles impacting the review process down stream?
- Review prioritized list of common issues and challenges
- Develop use cases for highest priority issues
- Identify additional issues, prioritize, and maintain log
- Create white paper and/or best practice documentation based on outcomes of discussions

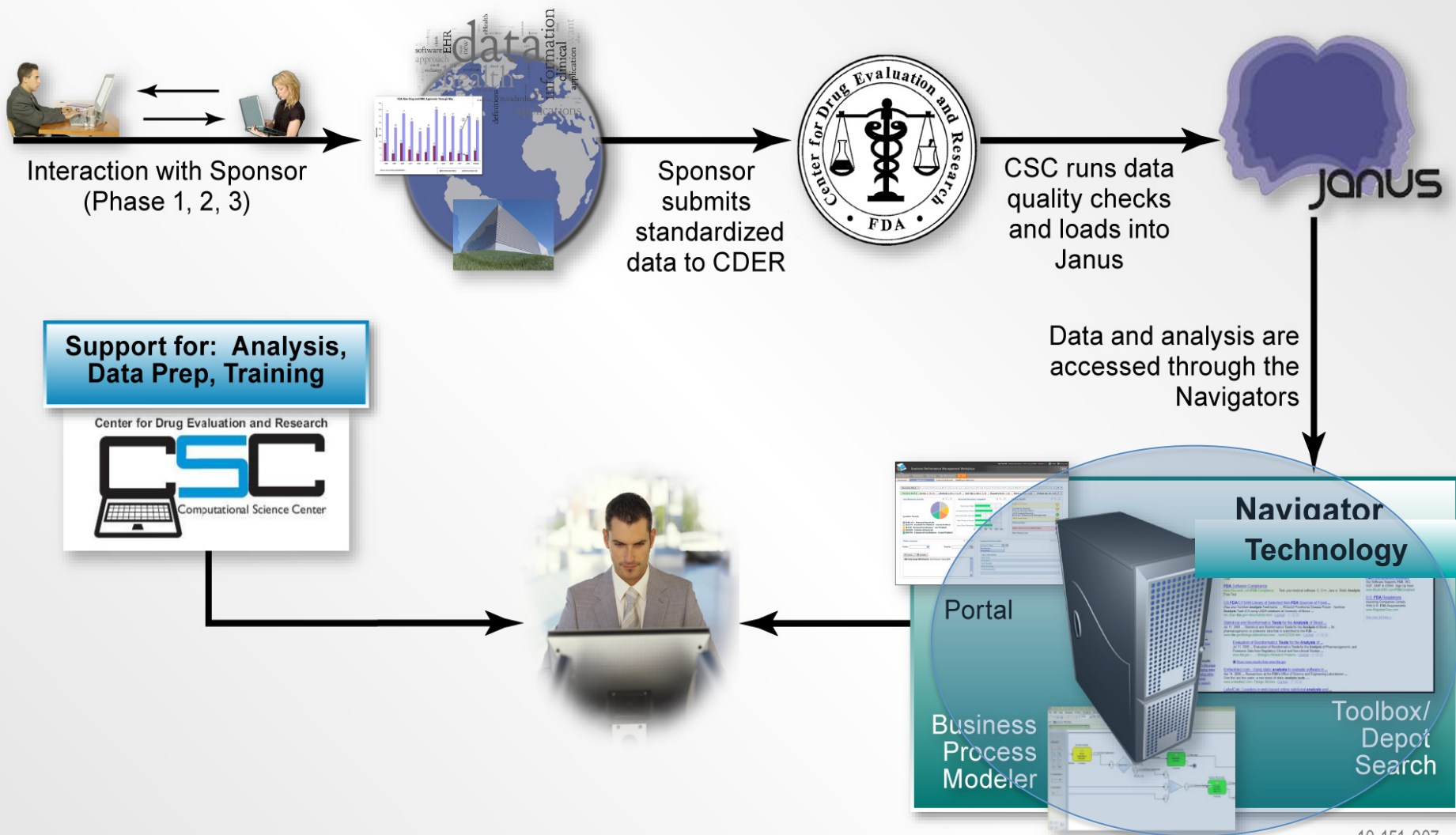


Focus of Work Group 4

- Numerous possible targets
- WG4 will discuss prioritization
 - Topics or issues related to the definitions and development of the CDISC models and terminology
 - Topics or issues related to the CDER Data Guide, previously referred to as the Reviewer's Guide
 - Topics or issues related to CDER's review of sponsor submissions
 - Topics or issues related to Industry implementation of CDISC data models

Work Group 5 — Development of Standard Scripts for

Analysis and Programming





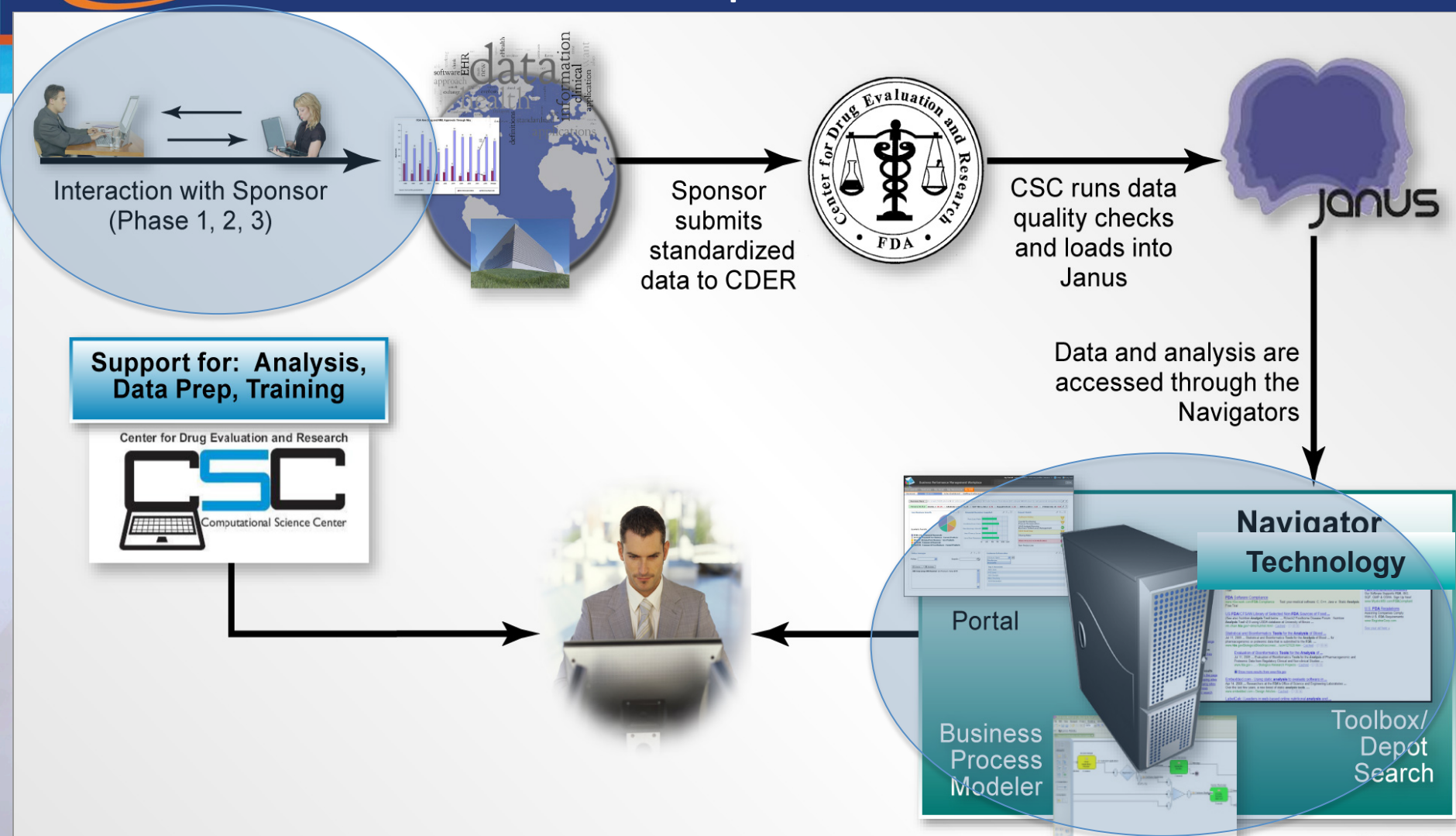
Work Group 5 – Development of Standard Scripts

- In past, FDA received data in many different formats
- Prevented development of library of standard scripts
- Goal of WG5:
 - identify potential standard scripts for data transformations and analyses across and/or within a therapeutic area and exploratory analysis
 - Develop environment to encourage collaborative problem solving where new methods are needed
- PhUSE Wiki
- Anticipated that this development work will identify additional data standards requirements

Work Group 6 — Non-Clinical Road-map and Impacts on



Implementation





Work Group 6 – Non-Clinical Road-map and Impacts on Implementation

- identify and prioritize needs and challenges for nonclinical data users, determine the overlap among stakeholders, and then develop the framework(s) for how to develop and pilot solutions that address the top priority needs.
- focus on specific implementation considerations for SEND
- develop a collaborative space to support implementers of the standard where resources, gaps, issues, challenges, and possible solutions are shared



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