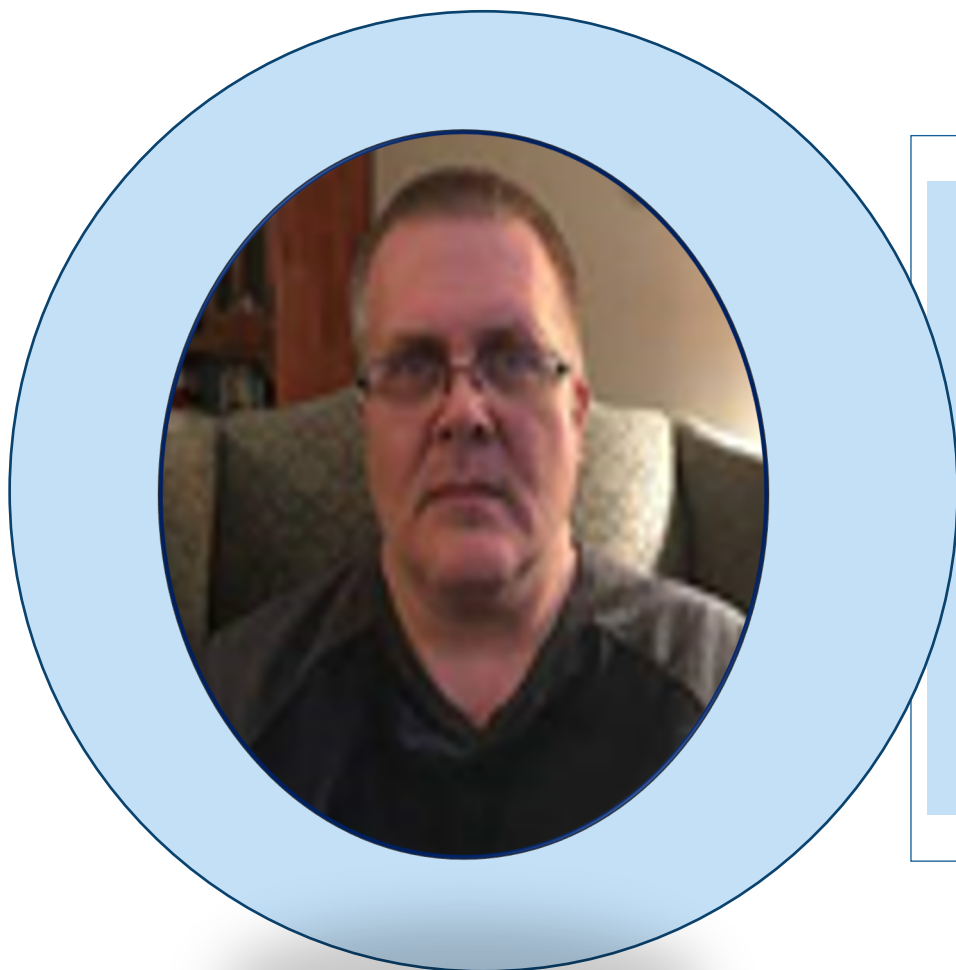


Staying Ahead of the Curve: The Future Direction of Modernization of Statistical Analytics

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**TransCelerate Role:
Sub Team Co-Lead of Modernization
of Statistical Analytics (MoA) Initiative**



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TransCelerate is a Not-for-Profit Entity Created to Foster Collaboration

Our mission is to collaborate across the global biopharmaceutical R&D community to identify, prioritize, design, and facilitate the implementation of solutions designed to drive the efficient, effective, and high-quality delivery of new medicines.

A Member-Driven Mission

Our Members are passionate about designing solutions to address R&D's most complex challenges related to clinical development & drug safety.

Membership is available to biopharmaceutical research and development organizations that engage in innovative discovery, development and manufacturing of new medicines*



* to be eligible for membership, companies must meet specified eligibility criteria.



What Was Heard

At the September TransCelerate MoA global webinar, we learned:

1. **Data quality**, with respect to the integrity of handling clinical data, **was the leading pain point**

2. Over 50% had begun **one of the stages of the MSA implementation journey**

3. The **most beneficial outcome of using the MSA Framework**, was that it **gave confidence** using modernized statistical analytics platform & creating opportunity to **obtain Health Authority (HA) endorsement**



Objectives of Modernization of Statistical Analytics (MoA) Initiative

The Current Landscape



Limited Regulatory Guidance

Specific analytical software is not required, but today's limited guidances create a significant obstacle to adopting new analytical tools



Increasing data volume

Emergence of electronic and digital data sources in clinical research has created a need for more advanced analytical capabilities



Greater availability of advanced technology

There are more advanced analytic capabilities that could be customized as fit for purpose in R&D

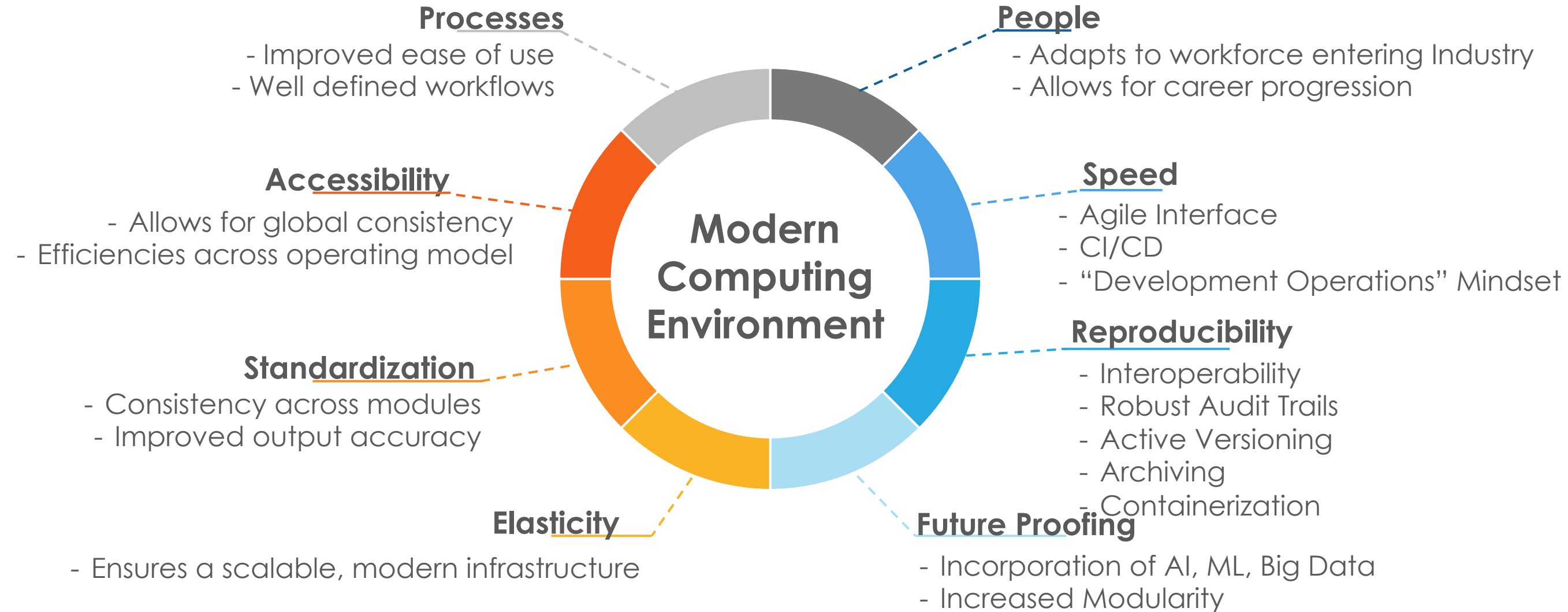
The Need

Organizations need to find a way to demonstrate to regulatory agencies that any analytical solution produces **credible results** and are suitable for use in the regulatory process.



Modernization of Statistical Analytics

Ideal State Components of a Modern Computing Environment



Three Core Principles for Regulatory Compliance & Implementation Success

Accuracy

The measure of **correctness** of software libraries that are used to generate results from an MSA environment – it is not the programs that developers write using those software libraries.

Traceability

Refers to the ability to **trace inputs to outputs**, with the main goal being to provide evidence to connect e-data, code, and environment to the final output that is produced.

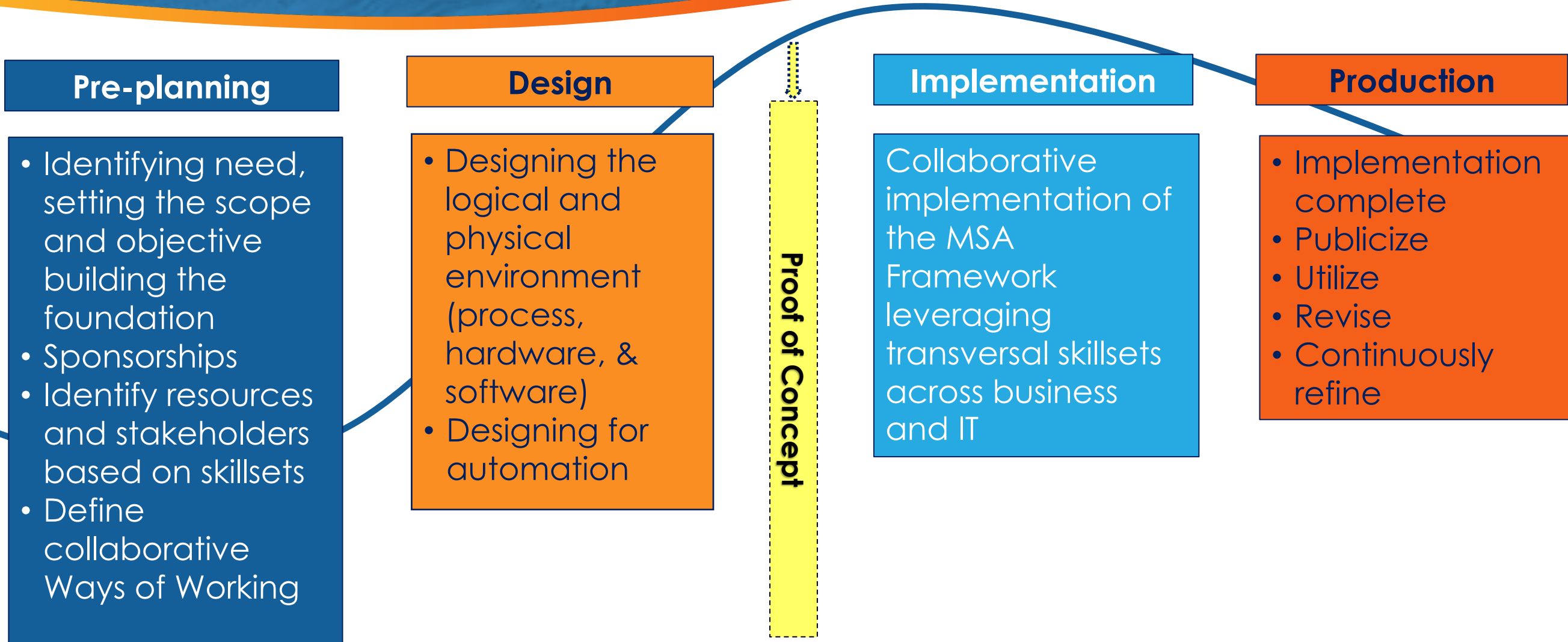


Reproducibility

If a statistical analysis output can be **recreated from the original dataset** along with the associated environment, including all artifacts and dependencies, it is **reproducible**. If an output is not reproducible, it is questionable whether results of the output can be trusted.



Implementation Model Of The MSA Framework



Case Study – Environment for Regulatory Submission*

*Note: TransCelerate does not endorse the use of any particular software

Case Study - Environment for Regulatory Submission*

Description/Scope

- Multi-server computational environment
- Contains 2 different types of installations – **Usual & Frozen** for submission related work

Framework Principles

Accuracy: Testing/validation

1. qualifications tests for packages considered in package assessment sufficiently tested
2. verification tests (reliability & correctness) for packages considered insufficiently tested

Reproducibility: Frozen installation with controlled execution

Traceability: Capturing script, context information, & standard out/error in the controlled execution

Intended Use

- Build a locked down environment to enable controlled execution
- Use a fixed set of packages in this environment to process & analyse clinical data to support submission work



Stakeholders

- Biostatisticians
- Statistical Analysts
- IT
- Business
- Operations



Case Study - Environment for Regulatory Submission*



Framework Benefits

- Gives confidence in using packages for regulatory submission
- Enables traditional quality control processes



Implementation Challenges

- Required collaboration & early engagement from stakeholders
- Communication is key – not all stakeholders are familiar with the environment
- The constant development of the environment



Efficiencies

- Implement analytic capabilities that require multiple solution providers to take advantage of emerging data for generating insights, decision making, & regulatory submission
- Guidance for a similar assessment for nontraditional software as established SOPs



Regulatory Submissions

- HAs have accepted trial design & statistical analyses using nontraditional software
- Sometimes have requested the programming code for review
- Willing to have an open discussion on what is the best methodology & tool



Case Study – Group Sequential Design*

*Note: TransCelerate does not endorse the use of any particular software

Case Study - Group Sequential Design*

Description/Scope

- Study design for group sequential studies
- Implemented SDLC to develop, document & validate designs
- User interface actively maintained and extended
- Expand universe of 'increasingly compliant' tools with increasing capabilities

Framework Principles

Accuracy

- Testing/validation
- Extensive unit testing providing a high-rate of coverage

Reproducibility

- Newly developed analytics platform with standard libraries
- Ease of use – User-friendly interface

Traceability

- Automate document & code generation using newly developed analytics
- SDLC process for consistent documentation

Intended Use

- Documenting bounds for statistical significance
- Response to regulatory questions concerning documentation of study designs
- Establish process for documenting designs



Stakeholders

- Statisticians
- Programmers
- Regulators
- Clinical study teams



Case Study - Group Sequential Design*



Framework Benefits

Mitigates risks

Unit testing & documentation

Guidance from metrics team considered in package updates

Maintaining code coverage with additions to the code base



Implementation Challenges

Large package with many functions

Package developed prior to evolution of current best practices



Efficiencies

Easy learning curve with graphical user interface

Consistent coding practices with automated code generation

Ability to save & restore designs to revisit assumptions & update at interim analyses

Report generation for documentation, review, auditing & reproducibility



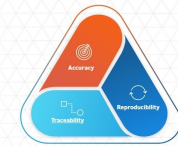
Regulatory Submissions

Protocols have been submitted using a standard tool

Process & testing updates improve documentation of regulatory compliance as we continue to update statistical analytical tools for study design documentation in protocols

*Note: TransCelerate does not endorse the use of any particular software





Engage With Us



- ⌵ Careers
- ⌵ Have an Idea? Let Us Know!
- ⌵ Give Feedback to an Initiative
- ⌵ Share Your Implementation Experience
- ⌵ Join Our Awareness & Implementation Community
- ⌵ Contact Us



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We'd love to hear from you!

Share feedback, comments or request more information using our MoA “Engage with Us Form”.



Give Feedback to an Initiative



Some of our initiatives are currently seeking feedback. If you have interest in providing input please select the initiative name from the dropdown below to complete their information request form. Please provide your email if you require a response to your inquiry. TransCelerate cannot respond through the feedback forms.

Initiative: Modernization of Statistical Analytics ⌵

Modernization of Statistical Analytics Feedback Form

TRANSCCELERATE INVITES YOU TO COMMENT ON THE MODERNIZATION OF STATISTICAL ANALYTICS (MSA) FRAMEWORK. PLEASE SHARE YOUR THOUGHTS AND RECOMMENDATIONS ON THIS SOLUTION PROPOSED BY TRANSCCELERATE.

NAME *

**Any Questions?
Thank You!**

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