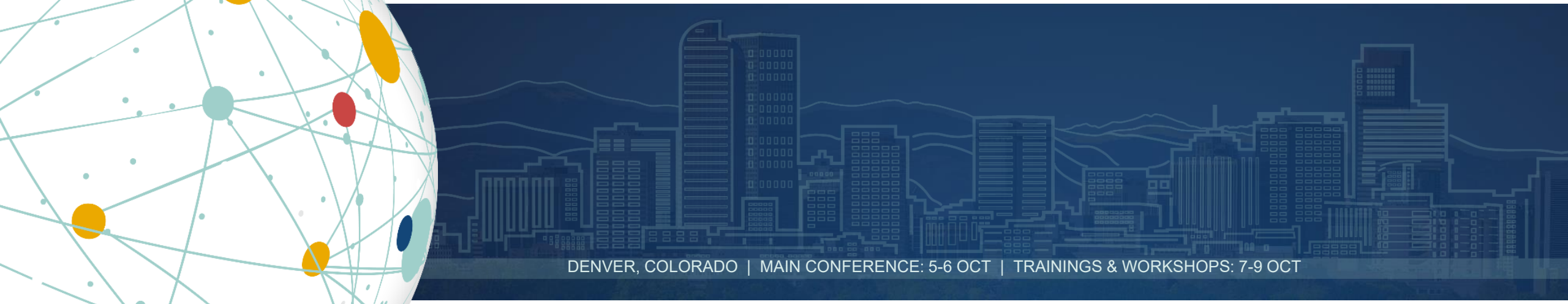


2026 US Interchange

THE FUTURE IS CONNECTED: STANDARDS AND AI POWERING DIGITAL TRANSFORMATION



Digital Therapeutic Area User Guides: A Modern Approach to Developing CDISC Standards

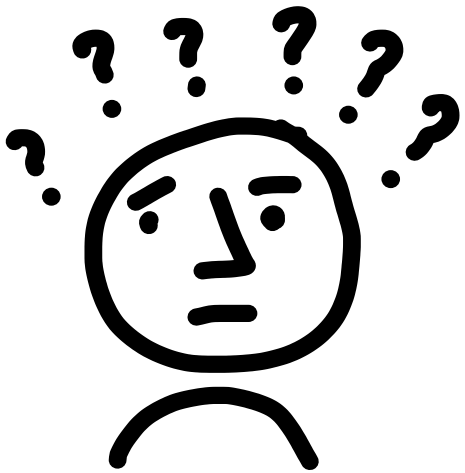
Bess LeRoy, Principal Director, CDISC

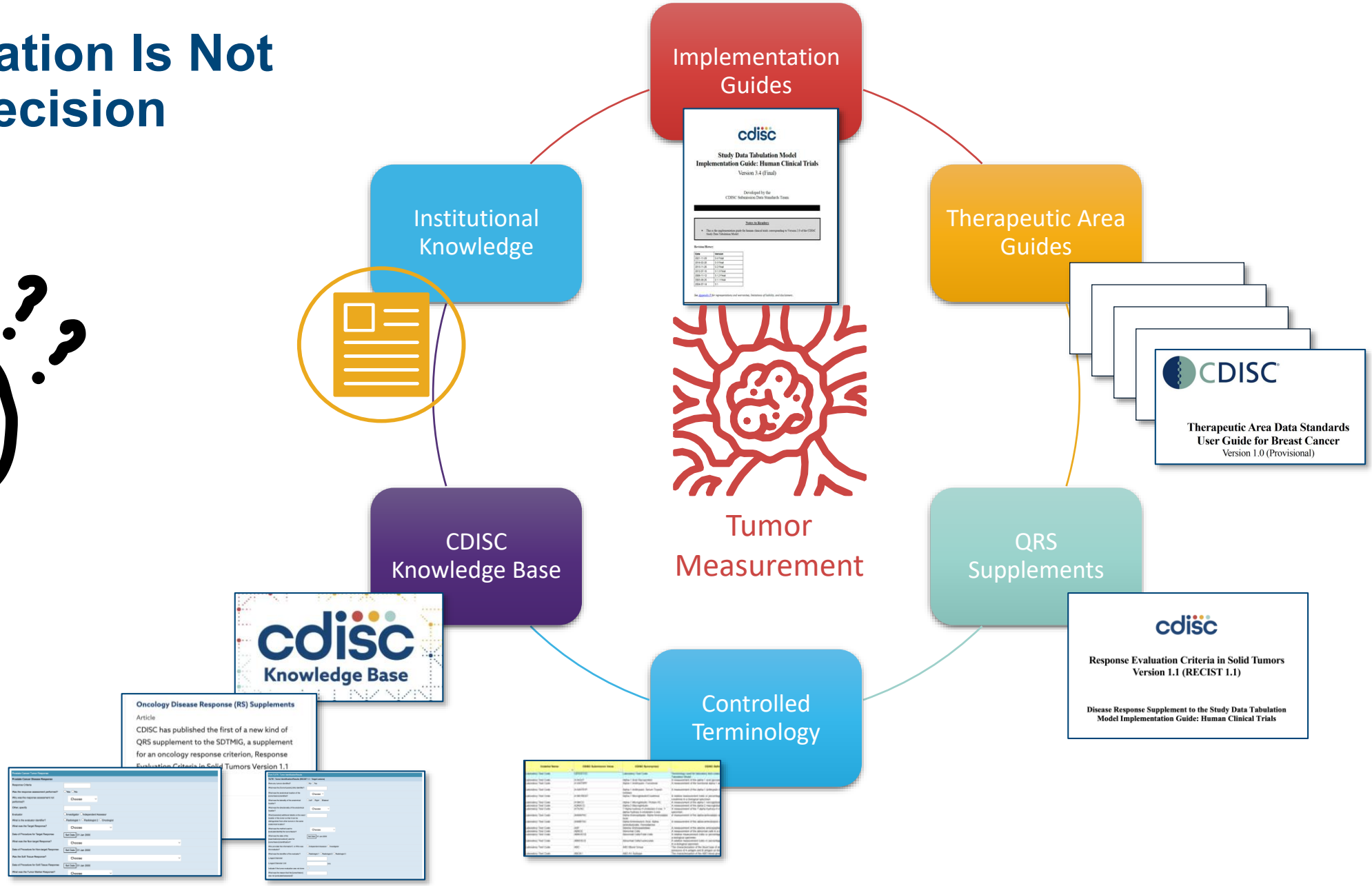
Agenda

- Representation vs Meaning
- The Role of Therapeutic Area User Guides
- Shifting to Meaning First With Biomedical Concepts
- Building Digital TAUGs, One Endpoint at a Time
- Art of the Possible: A PKD Registry Demo

Representation vs Meaning

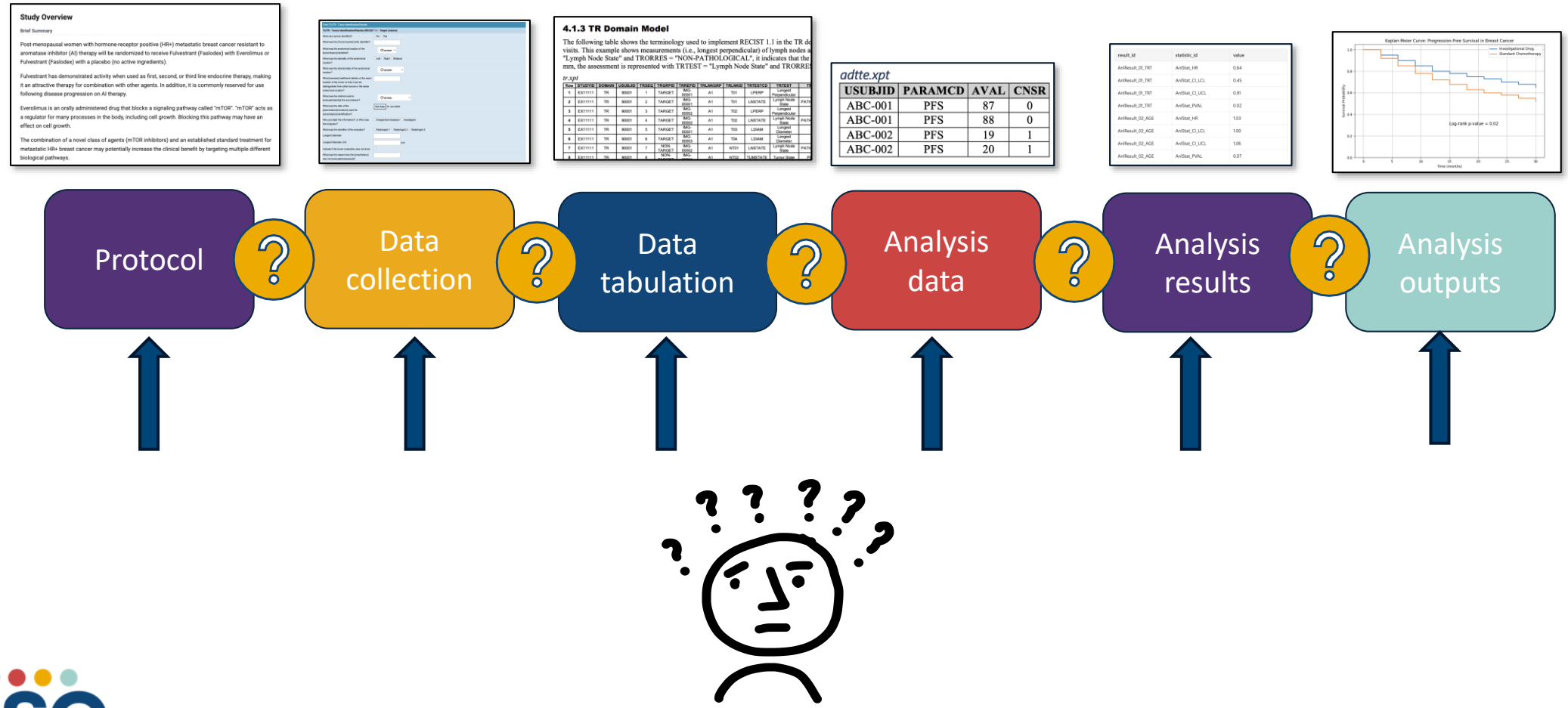
Representation Is Not a Single Decision





cdisc®

Meaning Does Not Explicitly Flow Across the Data Lifecycle



Consequences of Focusing on Representation First

Heavy reliance on expert interpretation at each stage with a focus on mapping

- Increases unnecessary variability
- Increases barriers to adoption

Context and intent are not explicitly linked from protocol through analysis

- Limits automation despite standards at each stage
- Challenges in establishing traceability
- Limits tool development

cdisc®

The Role of Therapeutic Area User Guides

Therapeutic Areas User Guides (TAUGs)

Autoimmune
- Psoriasis
- Rheumatoid Arthritis

Cardiovascular
- Cardiovascular
- Heart Failure
- QT Studies
- Traditional Chinese Medicine - Coronary Artery Disease-Angina

Endocrine
- Acute Kidney Injury
- Diabetes
- Diabetes Type 1 - Exercise and Nutrition
- Diabetes Type 1 - Pediatrics and Devices
- Diabetes Type 1 - Screening, Staging and Monitoring of Pre-clinical Type 1 Diabetes
- Diabetic Kidney Disease
- Dyslipidemia
- Kidney Transplant
- Polycystic Kidney Disease

Gastrointestinal
- CDAD
- Crohn's Disease

Infectious
- COVID-19
- Ebola
- Hepatitis C
- HIV
- Influenza
- Malaria
- Tuberculosis
- Virology

Mental Health
- Major Depressive Disorder
- Post Traumatic Stress Disorder
- Schizophrenia

Neurology
- Alzheimer's
- Huntington's Disease
- Multiple Sclerosis
- Parkinson's Disease
- Traumatic Brain Injury

Oncology
- Breast Cancer
- Colorectal Cancer
- Lung Cancer
- Pancreatic Cancer
- Prostate Cancer

Other
- Nutrition
- Pediatrics
- Traditional Chinese Medicine - Acupuncture

Rare Diseases
- Duchenne Muscular Dystrophy
- Rare Diseases

Respiratory
- Asthma
- COPD
- COVID-19

Treatments
- Pain
- Vaccines

TAUGs extend the CDISC Foundational Standards to represent data that pertains to specific disease areas

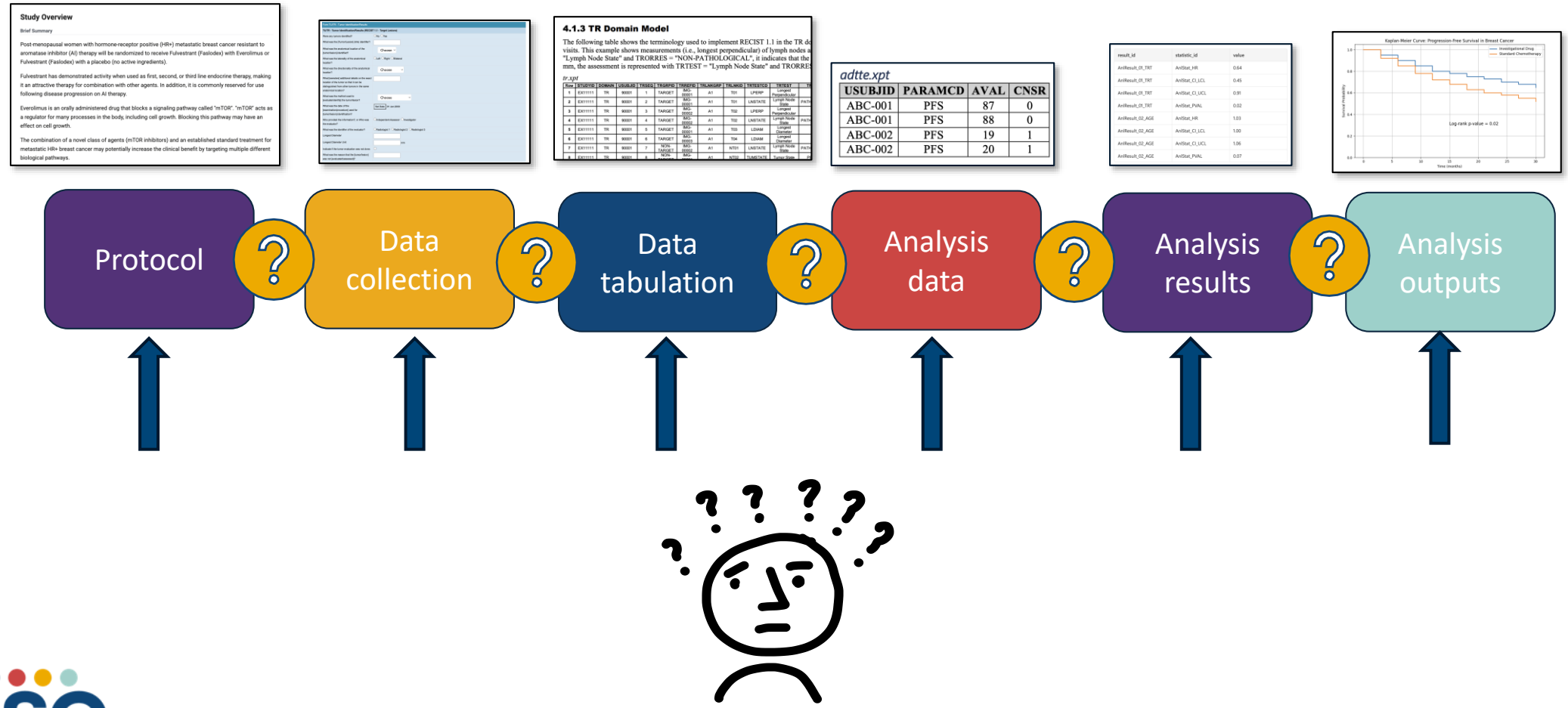
Increased focus on concepts

How TAUGs Evolved

2009-2010	• Concept Maps (*Cmaps) • SDTM Examples • Controlled Terminology
2011-2014	• Addition of CDASH CRF Examples
2015-2018	• Addition of ADaM Examples
2019-Present	• Full end-to-end standards alignment

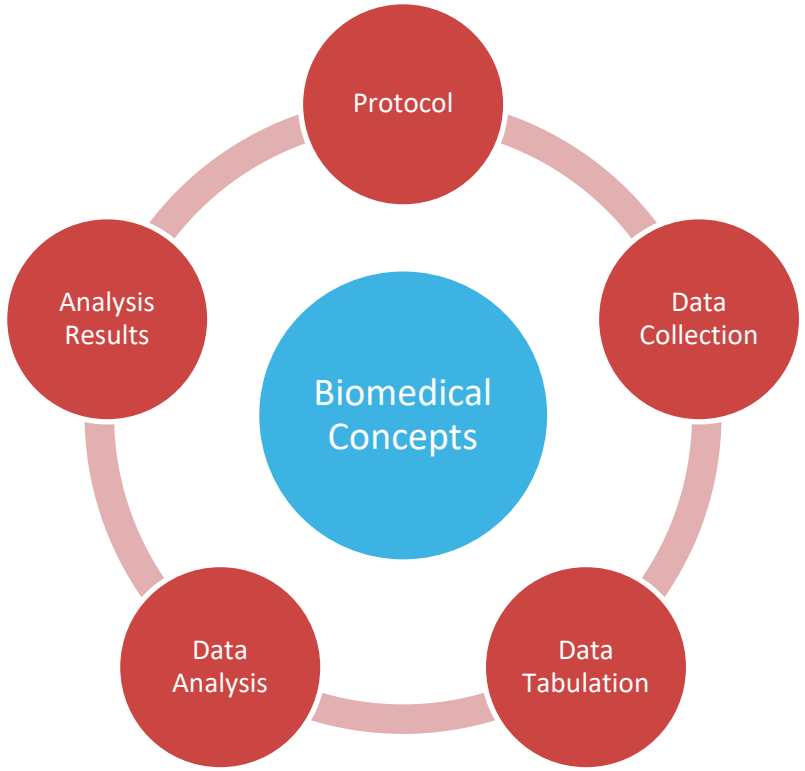
*https://cmap.ihmc.us

TAUGs Did Not Solve Meaning Flow



Shifting to Meaning First With Biomedical Concepts

Biomedical Concepts Anchor Clinical Meaning



Biomedical Concepts define clinical meaning once, and that meaning flows consistently into protocol design, data collection, data tabulation, analysis datasets, and analysis results

CDISC Machine Readable Biomedical Concepts

Conceptual Layer

- Clinical meaning
- Description of the concept in the real world
- Independent of a study and data standards

Implementation Layer

- Representation of the concept in a specific a Data Standard (e.g., CDASH, SDTM, FHIR, OMOP, etc.)



cdisc®

Creating Digital Assets Lays the Groundwork for Digitalization



Digitization

Analog to Digital

- Biomedical Concepts
  - Conceptual Layer
  - Implementation Layer
    - SDTM Specializations
    - CRF Specializations
    - Data Transfer Agreements
- Define.XML
- Analysis Concepts
- Implementation Examples

Digitalization uses Digital Assets to Improve How We Work

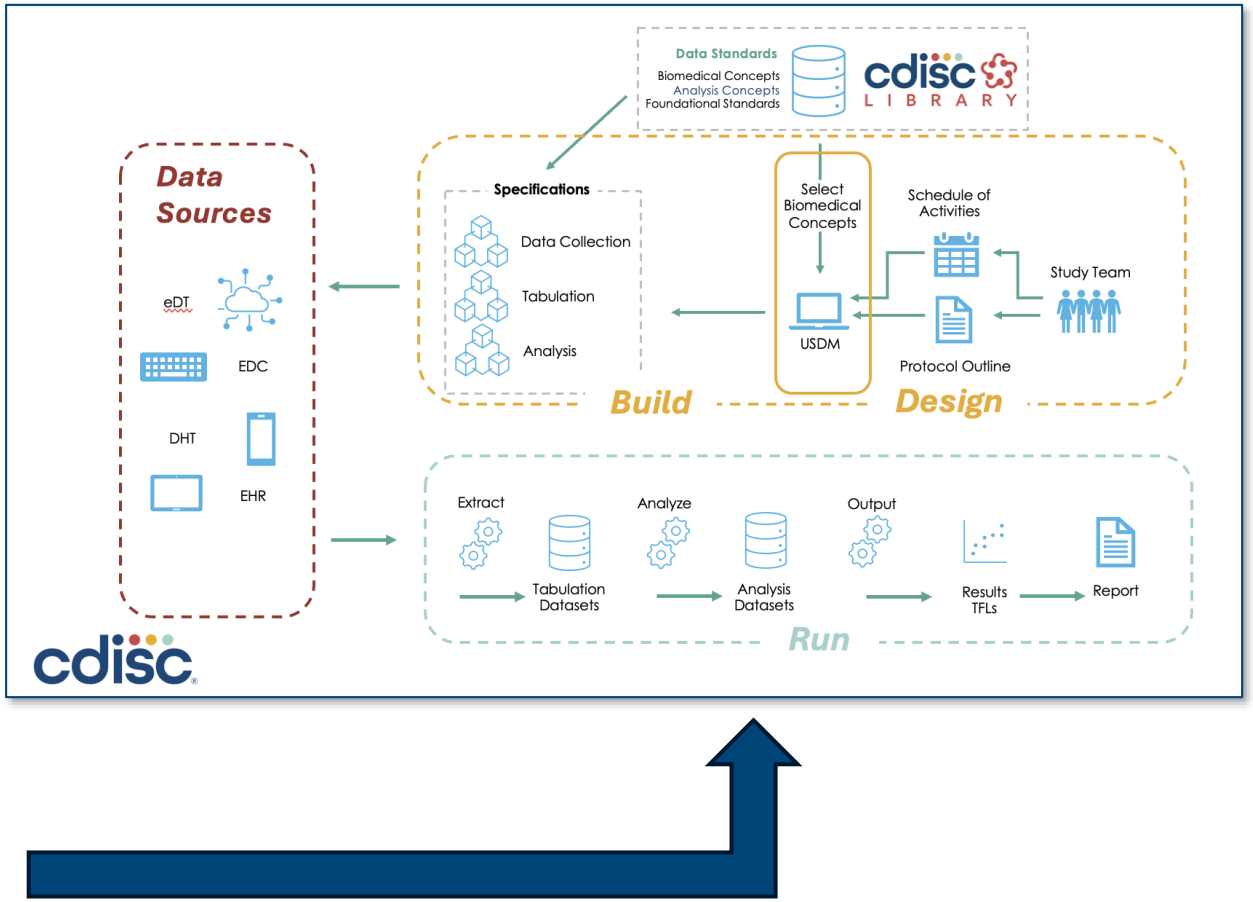
Biomedical Concepts

- Conceptual Layer
- Implementation Layer
  - SDTM Specializations
  - CRF Specializations
  - Data Transfer Agreements

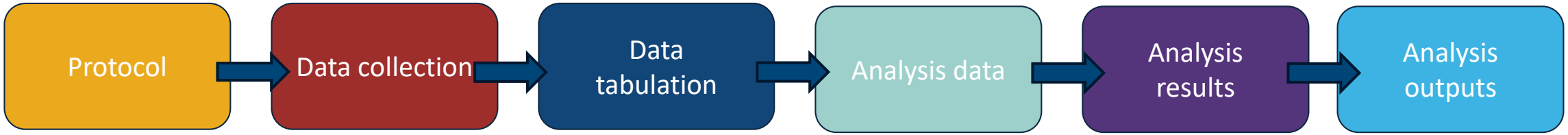
Define.XML

Analysis Concepts

Implementation Examples



Using Digital Assets to Enable Digitalization



Conceptual BC



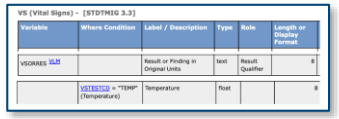
Schedule of Activities

BC CRF Implementation



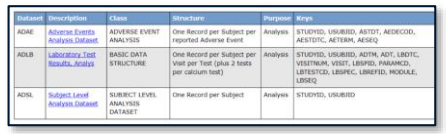
eCRF

BC SDTM Implementation



- *SDTM Define.XML*
- *Mapping Specifications*

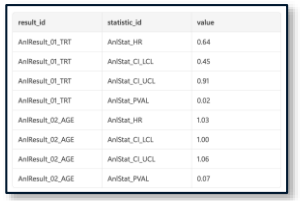
Analysis Concepts



- *ADaM Define.XML*
- *Mapping Specifications*

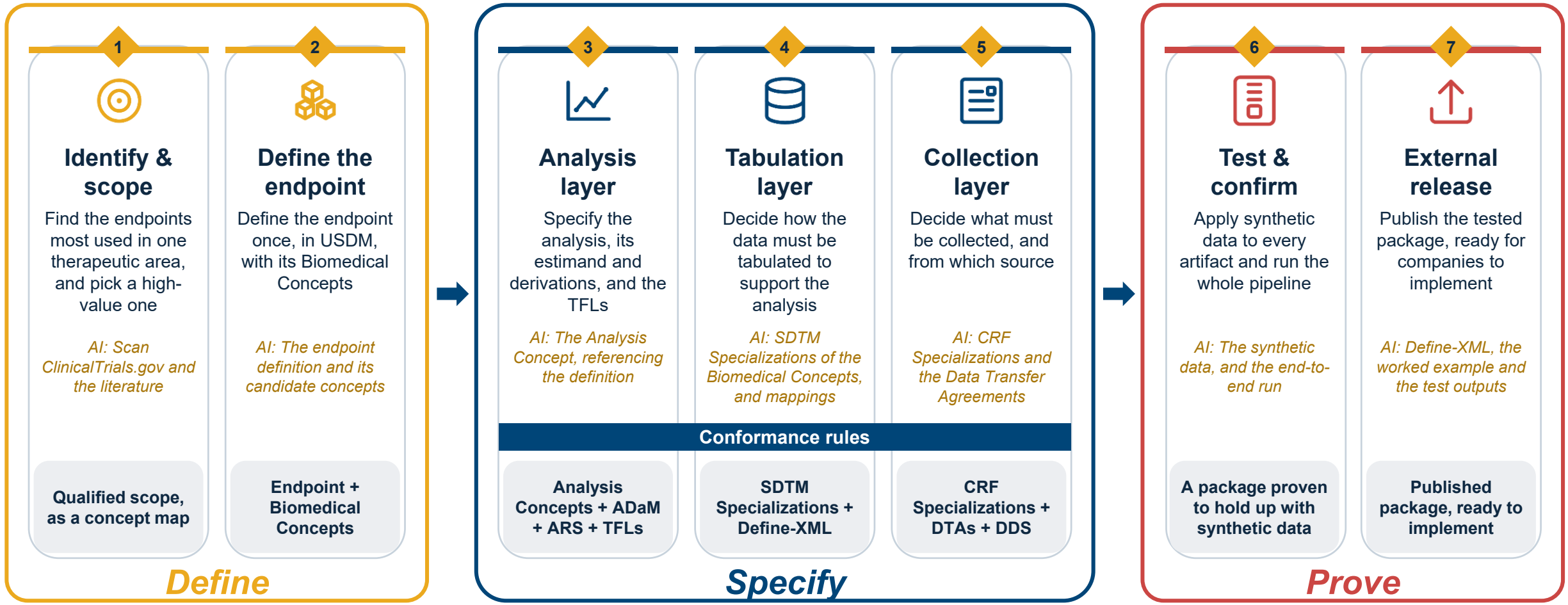


Analysis Results Metadata



Analysis Results Dataset

Building Meaning First Digital TAUGs, One Endpoint at a Time



From One Endpoint to a Therapeutic Area

01



One endpoint, specified end to end, with a small stage-specific expert group.

A published endpoint and a method we have run.

Prove it

02



The next endpoints in the same area, reusing the concepts the first one established.

Cycle time falls with each pass.

Repeat it

03



Enough endpoints to assemble the therapeutic area's digital TAUG.

The first genuinely digital TAUG.

Complete it

04



The shared concepts carry into the next therapeutic area.

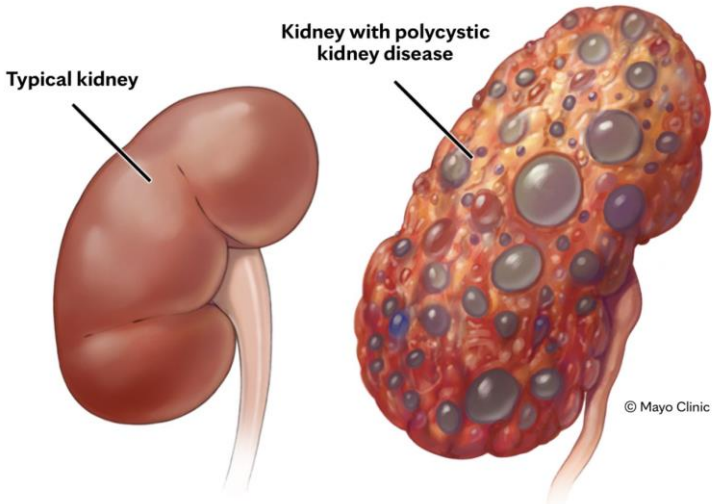
Each new area starts part-built, not from zero.

Extend it

Art of the Possible:
A PKD Registry Demo

Configuring Analysis-Informed Standards for a PKD Registry

- I am a researcher studying Polycystic Kidney Disease (PKD), a genetic disorder affecting kidney function that currently has no cure
- My is goal is to set up a disease registry to collect data in an observational setting to understand disease characteristics, disease progression, treatment patterns, and outcomes over time
- My data collection plan is flexible and evolving and while I have hypotheses I would like test, my analysis plan is not fixed
- I am interested in using CDISC Standards but as an academic researcher, I don't have the same years of experience with data standards as someone working in the pharmaceutical industry



Source: https://mcpress.mayoclinic.org/polycystic-kidney-disease

CDISC
Digital TAUG Prototype

DIGITAL TAUGS

Study Standards Configuration

1. Select Therapeutic Area
2. Select Disease
3. Select Concepts
4. Configure Concepts
5. Schedule of Activities
6. Analyses
7. Analysis Readiness
8. Outputs

Study Snapshot

TA: —

Disease: —

Concepts: **0**

Analyses: **0**

CDISC DIGITAL TAUGS

Study Standards Configuration

Step 1 of 8

Select Therapeutic Area

Choose a therapeutic area to scope the study standards flow.

Search therapeutic areas

- Oncology
- Neurology
- Cardiovascular
- Renal/Nephrology
- Infectious Diseases
- Gastroenterology

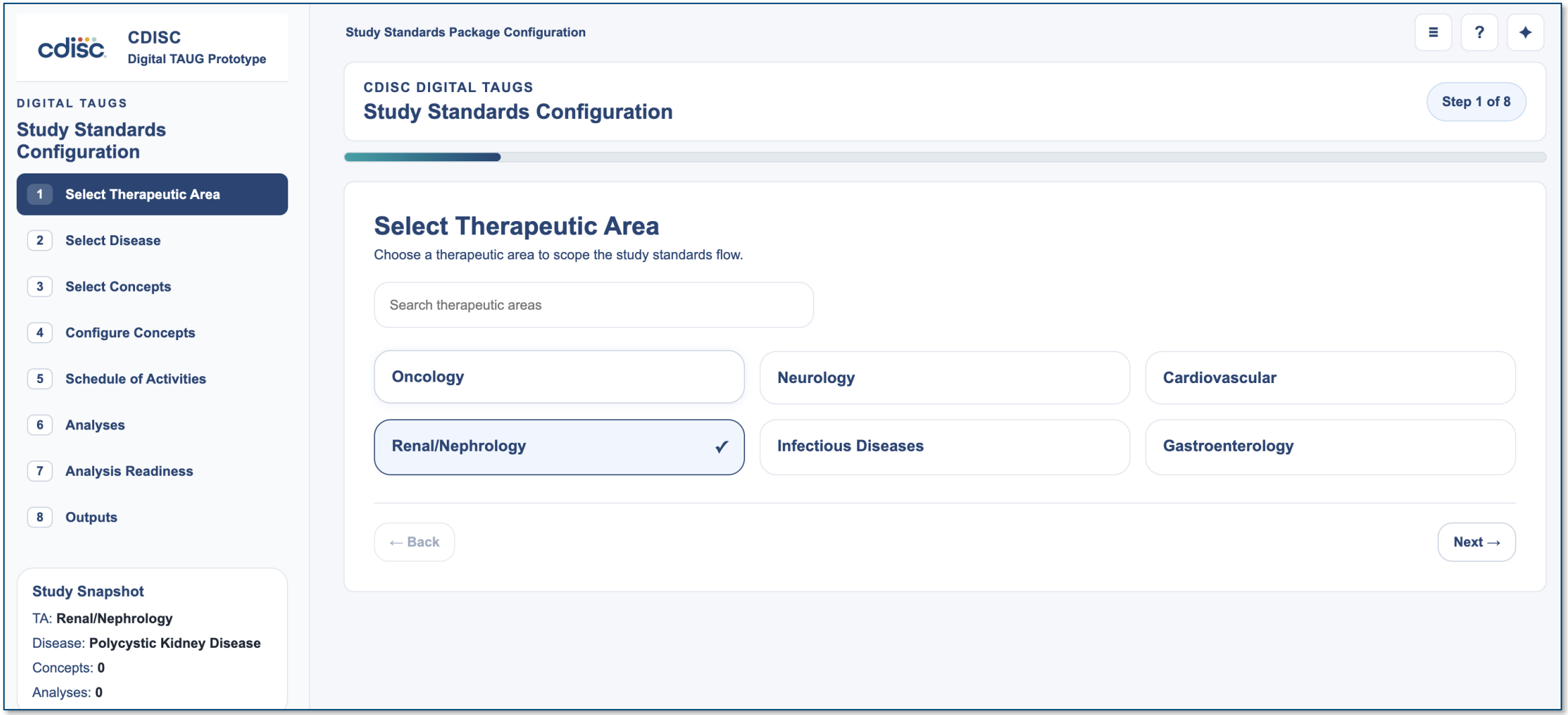
← Back

Next →

Illustrative – Not Production Tool

Thank You!




CDISC
Digital TAUG Prototype
DIGITAL TAUGS
Study Standards Configuration
1 Select Therapeutic Area
2 Select Disease
3 Select Concepts
4 Configure Concepts
5 Schedule of Activities
6 Analyses
7 Analysis Readiness
8 Outputs
Study Snapshot
TA: Renal/Nephrology
Disease: Polycystic Kidney Disease
Concepts: 0
Analyses: 0
Study Standards Package Configuration
CDISC DIGITAL TAUGS
Study Standards Configuration
Step 1 of 8
Select Therapeutic Area
Choose a therapeutic area to scope the study standards flow.
Search therapeutic areas
Oncology
Neurology
Cardiovascular
Renal/Nephrology
Infectious Diseases
Gastroenterology
← Back
Next →

CDISC
Digital TAUG Prototype

DIGITAL TAUGS

Study Standards Configuration

- ✓ Select Therapeutic Area
- 2 Select Disease
- 3 Select Concepts
- 4 Configure Concepts
- 5 Schedule of Activities
- 6 Analyses
- 7 Analysis Readiness
- 8 Outputs

Study Snapshot

TA: **Renal/Nephrology**
Disease: **Polycystic Kidney Disease**
Concepts: **0**
Analyses: **0**

CDISC DIGITAL TAUGS

Study Standards Configuration

Step 2 of 8

Select Disease

Filtered by the selected therapeutic area.

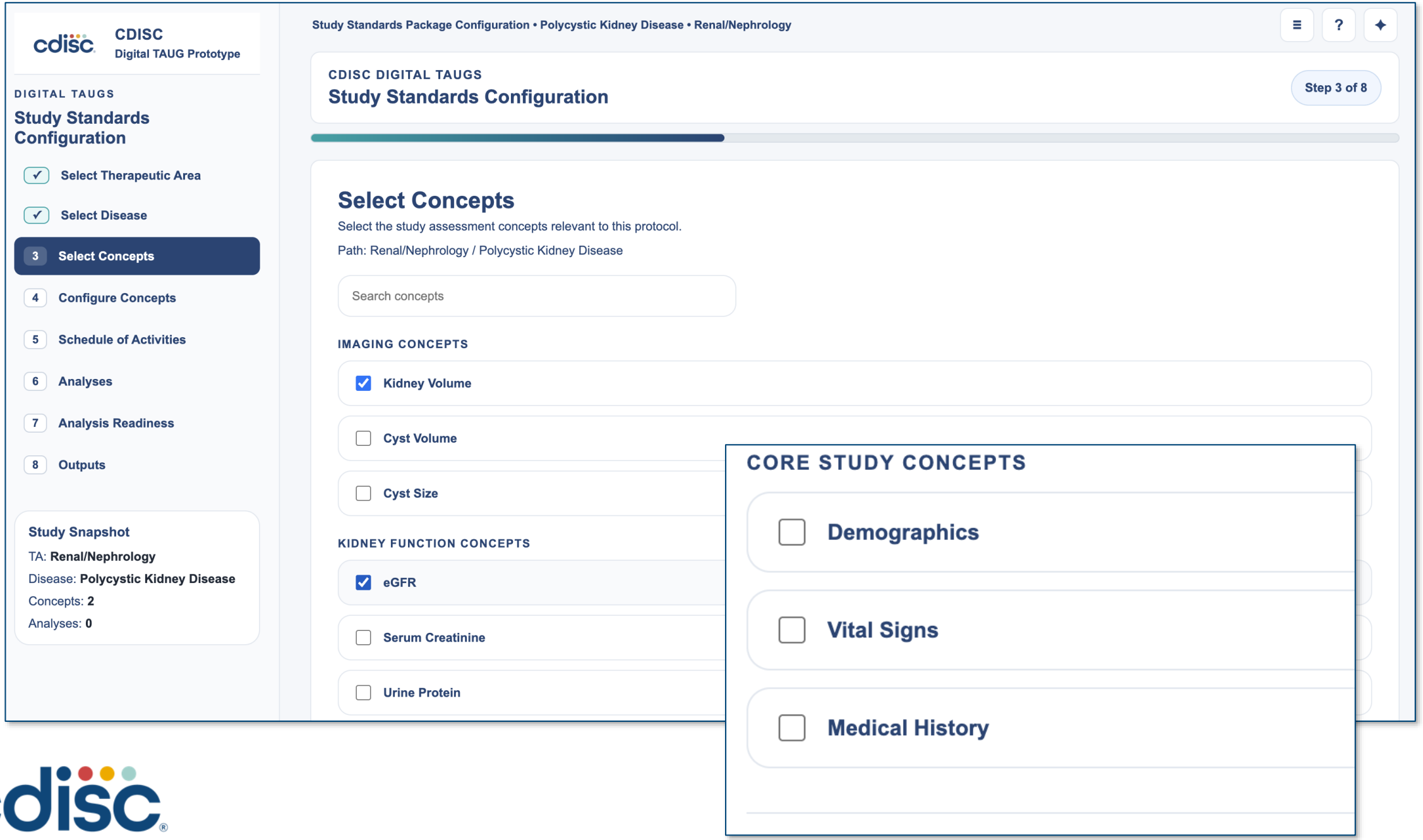
Therapeutic Area: **Renal/Nephrology**

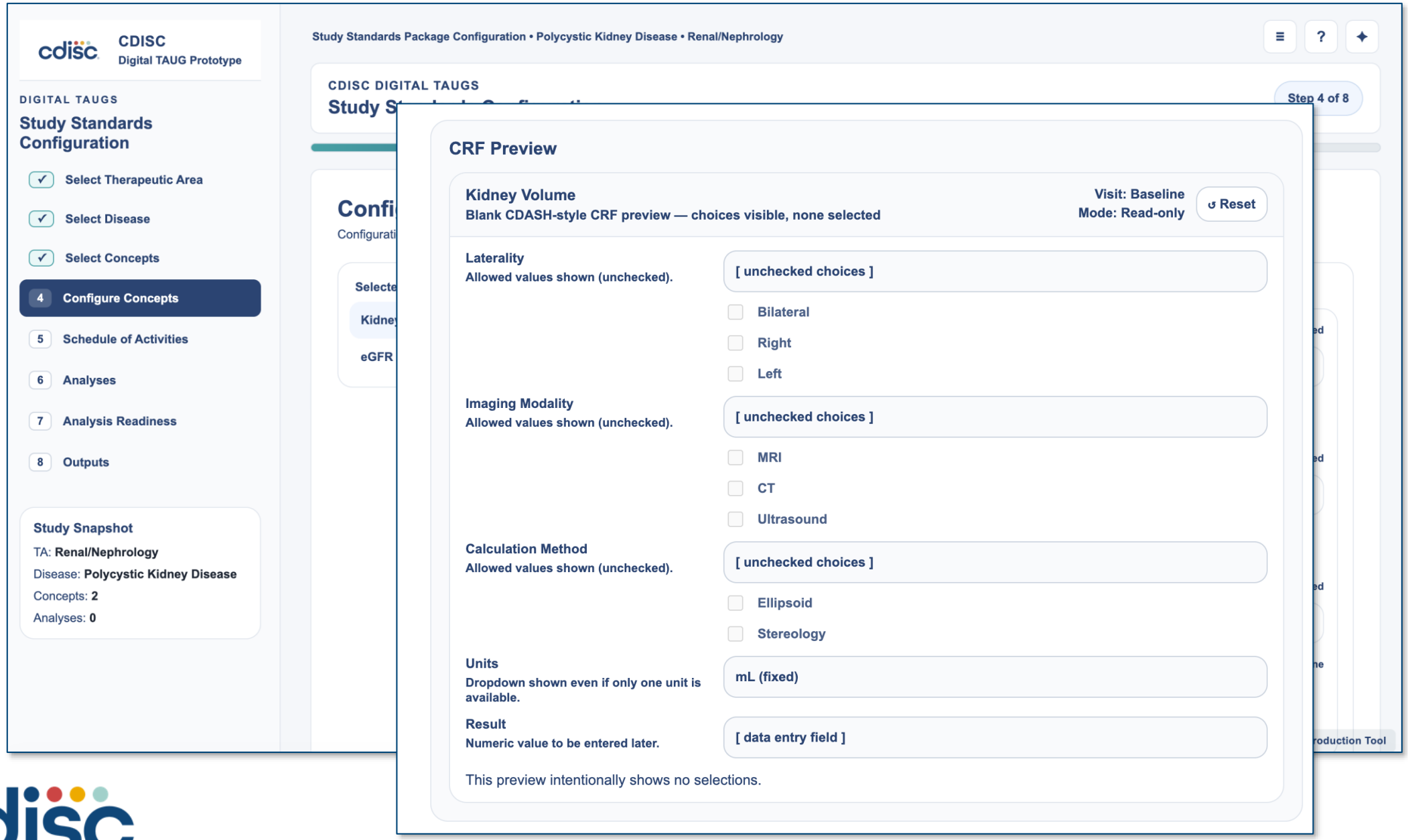
Search diseases

- **Chronic Kidney Disease**
- **Acute Kidney Injury**
- **Polycystic Kidney Disease**
- **Kidney Transplant**

← Back

Next →


CDISC
Digital TAUG Prototype
DIGITAL TAUGS
Study Standards Configuration
Select Therapeutic Area
Select Disease
3 Select Concepts
4 Configure Concepts
5 Schedule of Activities
6 Analyses
7 Analysis Readiness
8 Outputs
Study Snapshot
TA: Renal/Nephrology
Disease: Polycystic Kidney Disease
Concepts: 2
Analyses: 0
Study Standards Package Configuration • Polycystic Kidney Disease • Renal/Nephrology
CDISC DIGITAL TAUGS
Study Standards Configuration
Step 3 of 8
Select Concepts
Select the study assessment concepts relevant to this protocol.
Path: Renal/Nephrology / Polycystic Kidney Disease
Search concepts
IMAGING CONCEPTS
Kidney Volume
Cyst Volume
Cyst Size
KIDNEY FUNCTION CONCEPTS
eGFR
Serum Creatinine
Urine Protein
CORE STUDY CONCEPTS
Demographics
Vital Signs
Medical History


CDISC
Digital TAUG Prototype
DIGITAL TAUGS
Study Standards Configuration
Select Therapeutic Area
Select Disease
Select Concepts
4 Configure Concepts
5 Schedule of Activities
6 Analyses
7 Analysis Readiness
8 Outputs
Study Snapshot
TA: Renal/Nephrology
Disease: Polycystic Kidney Disease
Concepts: 2
Analyses: 0
Study Standards Package Configuration • Polycystic Kidney Disease • Renal/Nephrology
CDISC DIGITAL TAUGS
Step 4 of 8
CRF Preview
Kidney Volume
Blank CDASH-style CRF preview — choices visible, none selected
Visit: Baseline
Mode: Read-only
Reset
Laterality
Allowed values shown (unchecked).
[unchecked choices]
Bilateral
Right
Left
Imaging Modality
Allowed values shown (unchecked).
[unchecked choices]
MRI
CT
Ultrasound
Calculation Method
Allowed values shown (unchecked).
[unchecked choices]
Ellipsoid
Stereology
Units
Dropdown shown even if only one unit is available.
mL (fixed)
Result
Numeric value to be entered later.
[data entry field]
This preview intentionally shows no selections.

CDISC
Digital TAUG Prototype

DIGITAL TAUGS

Study Standards Configuration

- ✓ Select Therapeutic Area
- ✓ Select Disease
- ✓ Select Concepts
- ✓ Configure Concepts
- 5 Schedule of Activities
- 6 Analyses
- 7 Analysis Readiness
- 8 Outputs

Study Snapshot

TA: **Renal/Nephrology**
Disease: **Polycystic Kidney Disease**
Concepts: **2**
Analyses: **0**

CDISC DIGITAL TAUGS

Study Standards Configuration

Step 5 of 8

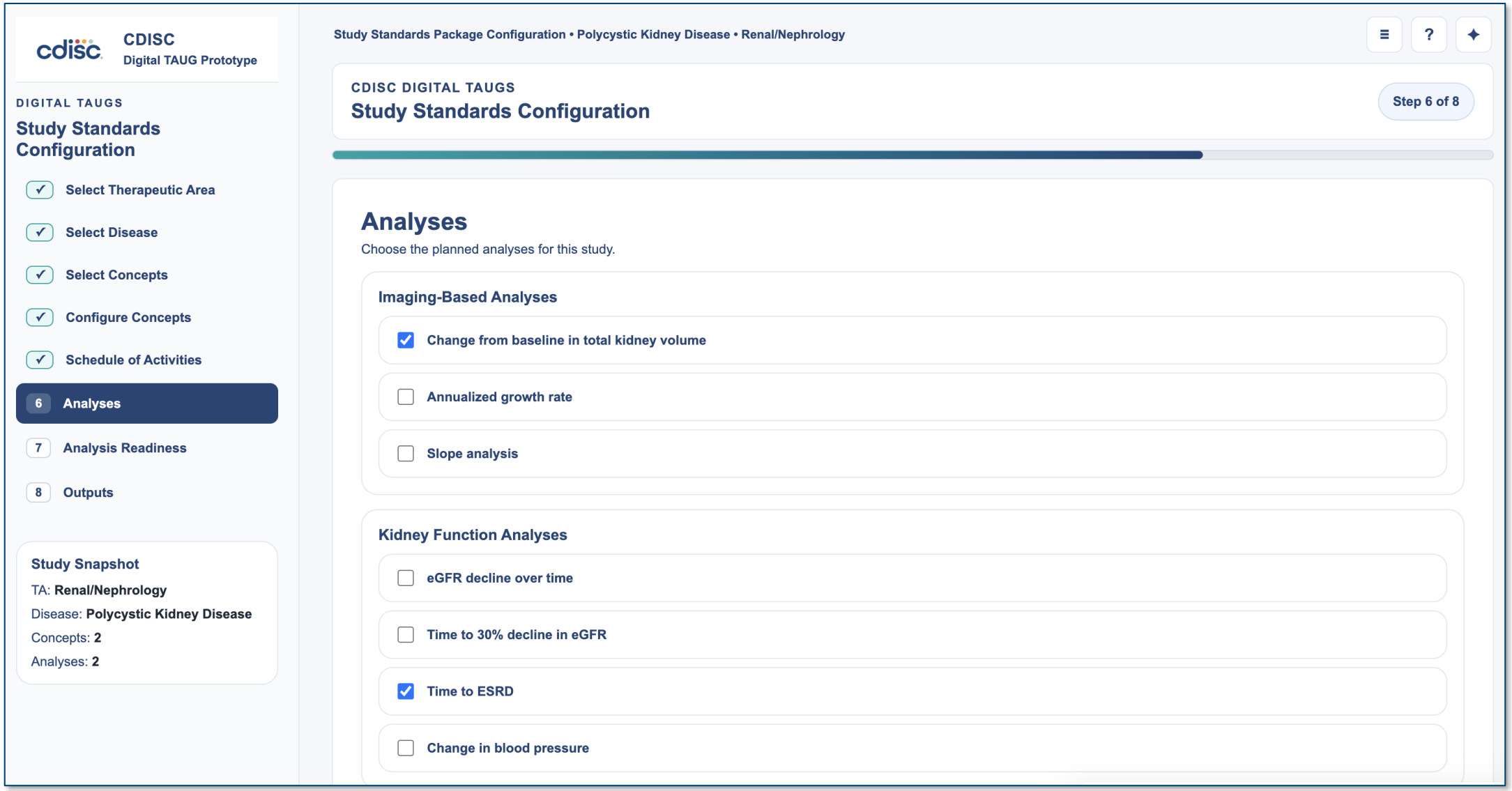
Schedule of Activities

Fixed visit structure with checkbox scheduling per concept.

CONCEPT	SCREENING	BASELINE	MONTH 3	MONTH 6
Kidney Volume	☐	☐	☐	☐
eGFR	☐	☐	☐	☐

← Back

Next →


CDISC
Digital TAUG Prototype
DIGITAL TAUGS
Study Standards Configuration
Select Therapeutic Area
Select Disease
Select Concepts
Configure Concepts
Schedule of Activities
6 Analyses
7 Analysis Readiness
8 Outputs
Study Snapshot
TA: Renal/Nephrology
Disease: Polycystic Kidney Disease
Concepts: 2
Analyses: 2
Study Standards Package Configuration • Polycystic Kidney Disease • Renal/Nephrology
CDISC DIGITAL TAUGS
Study Standards Configuration
Step 6 of 8
Analyses
Choose the planned analyses for this study.
Imaging-Based Analyses
Change from baseline in total kidney volume
Annualized growth rate
Slope analysis
Kidney Function Analyses
eGFR decline over time
Time to 30% decline in eGFR
Time to ESRD
Change in blood pressure

CDISC
Digital TAUG Prototype

DIGITAL TAUGS

Study Standards Configuration

- ✓ Select Therapeutic Area
- ✓ Select Disease
- ✓ Select Concepts
- ✓ Configure Concepts
- ✓ Schedule of Activities
- ✓ Analyses
- 7 **Analysis Readiness**
- 8 Outputs

Study Snapshot

TA: **Renal/Nephrology**
Disease: **Polycystic Kidney Disease**
Concepts: **2**
Analyses: **2**

CDISC DIGITAL TAUGS
Study Standards Configuration

Step 7 of 8

Analysis Readiness

Rule-based readiness results for the selected analyses.

✓ Change from baseline in total kidney volume

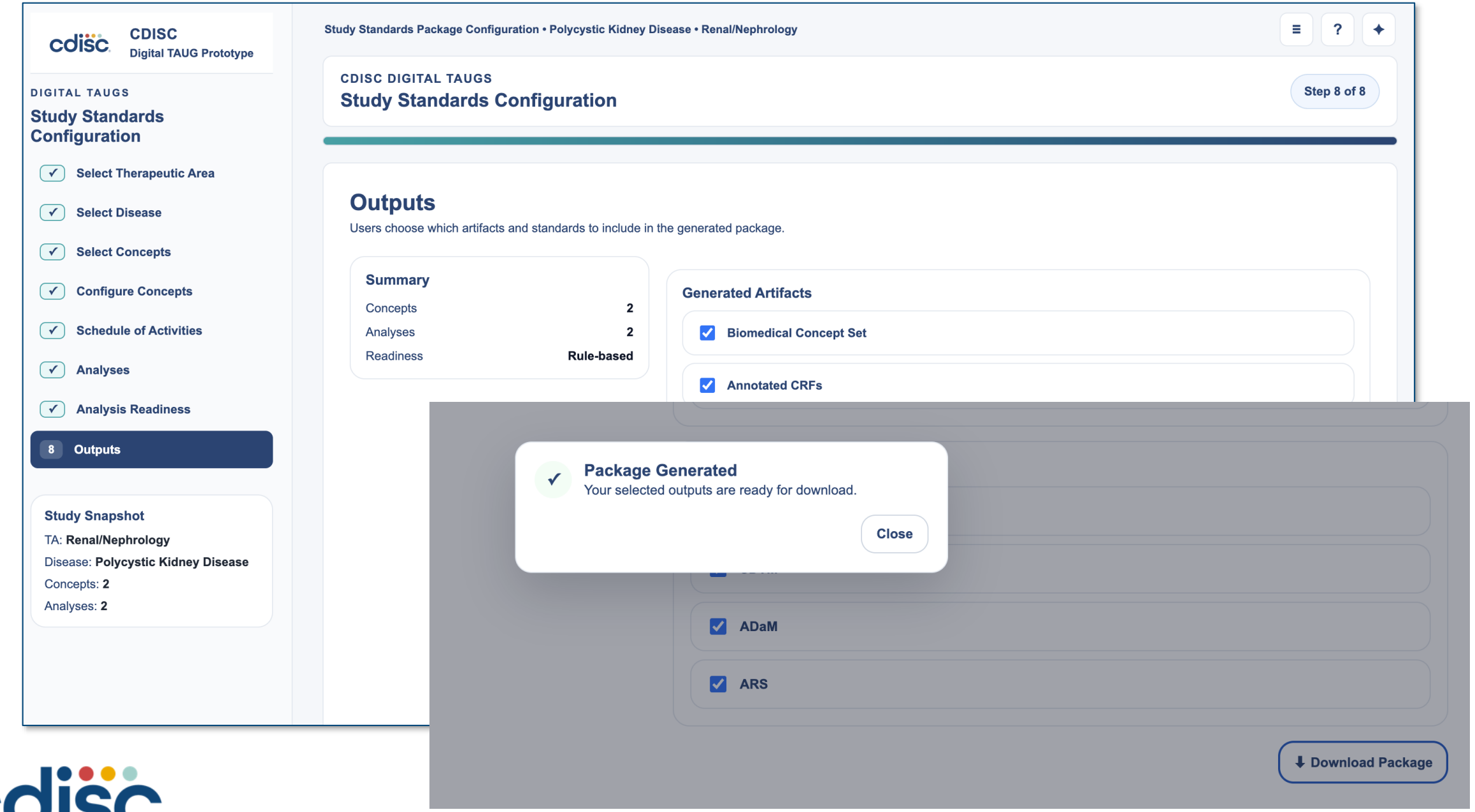
- ✓ Baseline visit defined
- ✓ Longitudinal timepoints available
- ✓ Image modality specified
- ✓ Units standardized (mL)

⚠ Time to ESRD

- ! ESRD event definition not selected
- ! Event data not scheduled
- ! Disposition tracking not included

← Back

Next →


CDISC
Digital TAUG Prototype
DIGITAL TAUGS
Study Standards Configuration
Select Therapeutic Area
Select Disease
Select Concepts
Configure Concepts
Schedule of Activities
Analyses
Analysis Readiness
8 Outputs
Study Snapshot
TA: Renal/Nephrology
Disease: Polycystic Kidney Disease
Concepts: 2
Analyses: 2
Study Standards Package Configuration • Polycystic Kidney Disease • Renal/Nephrology
CDISC DIGITAL TAUGS
Study Standards Configuration
Step 8 of 8
Outputs
Users choose which artifacts and standards to include in the generated package.
Summary
Concepts 2
Analyses 2
Readiness Rule-based
Generated Artifacts
Biomedical Concept Set
Annotated CRFs
Package Generated
Your selected outputs are ready for download.
Close
ADaM
ARS
Download Package