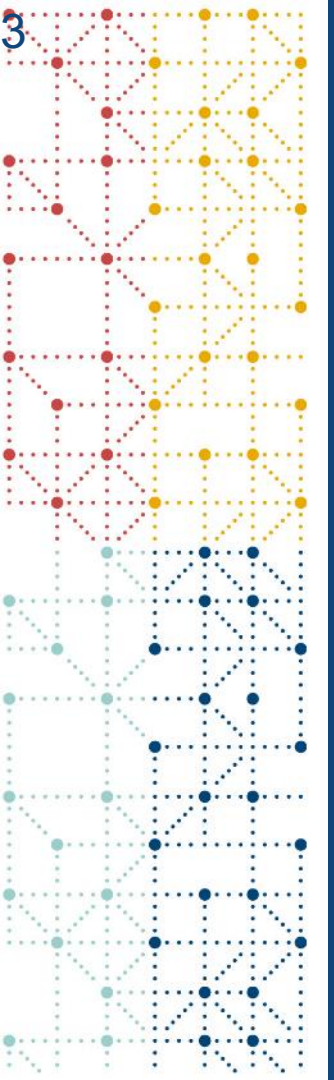
Pediatrics User Guide – Summary of Scoping

John Owen, Head of Partnerships & Development, CDISC
Richard Marshall, Lead Developer, CDISC

TUE 21 SEP
11:00AM-11:45AM ET

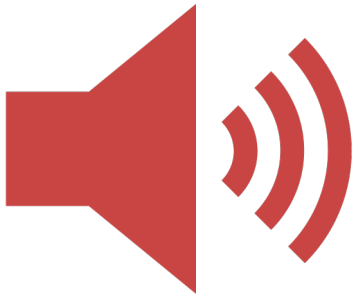
Today's Agenda

1. Housekeeping
2. Feature Presentation + Q&A
3. Upcoming Learning Opportunities & Events



Housekeeping

Housekeeping

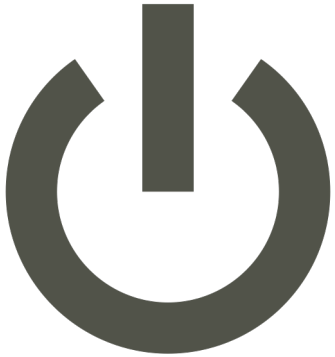


You will remain on **mute**

Housekeeping

Submit questions at any time via the Questions tool on your GoToWebinar app

Housekeeping



Audio issues?

Shut down & restart GoToWebinar app

Housekeeping

A recording of this webinar and the slides will be available in the **Members Only** section of CDISC website

Today's Presenters

John Owen

Head of Partnerships & Development

CDISC

Richard Marshall

Lead Developer

CDISC

Pediatrics User Guide
Scoping Summary Webinar

John Owen, Head, PMO, CDISC
Richard Marshall, Lead Developer, CDISC
2021-09-21

- Global Clinical Data Standards Development Organization
- Founded in 1997 (all volunteers)
- Incorporated in 2000 as a non-profit organization

Why is CDISC Important?

- By bringing together a global community of experts to develop and advance data standards of the highest quality, CDISC creates clarity in clinical research.
- Together, we enable the accessibility, interoperability, and reusability of data for more meaningful and efficient research that has greater impact on global health.

Why Standardize with CDISC?

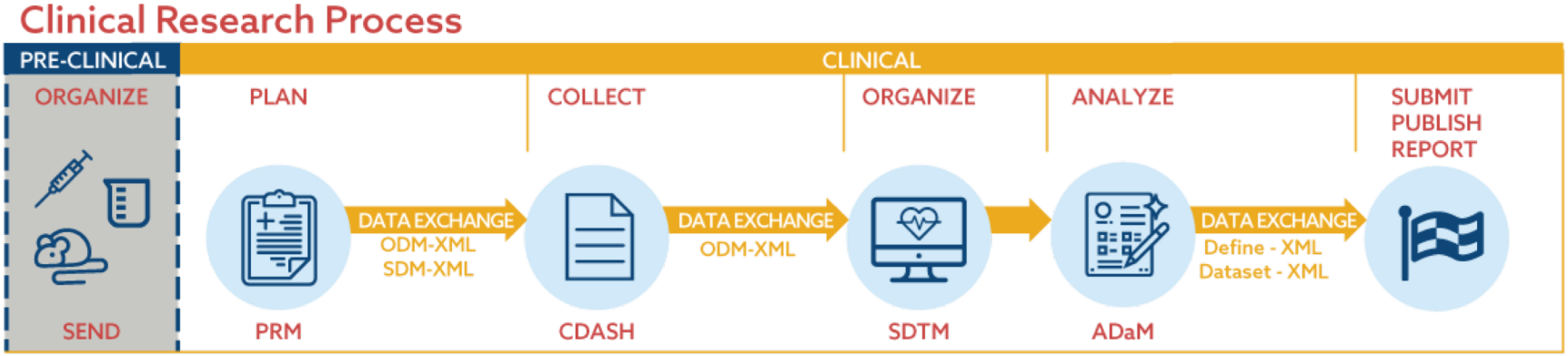
Global standard for all types of clinical research

- Create familiarity - know where to find things, understand what they mean
- Allow software systems to be built on CDISC
- Facilitate meaningful data sharing (academia, public health)
- Connect to EHR data through BRIDG
- Widely adopted due to requirements by global regulatory agencies (FDA, PMDA and NMPA) and endorsement by others (CFDA, EMA)
- Data sharing accelerates research progress

Developed and maintained through open consensus-based process

- Developed by subject matter experts
- Widely vetted during open public review, training and implementation
- Feedback from implementation informs further development

CDISC Standards in the Research Process



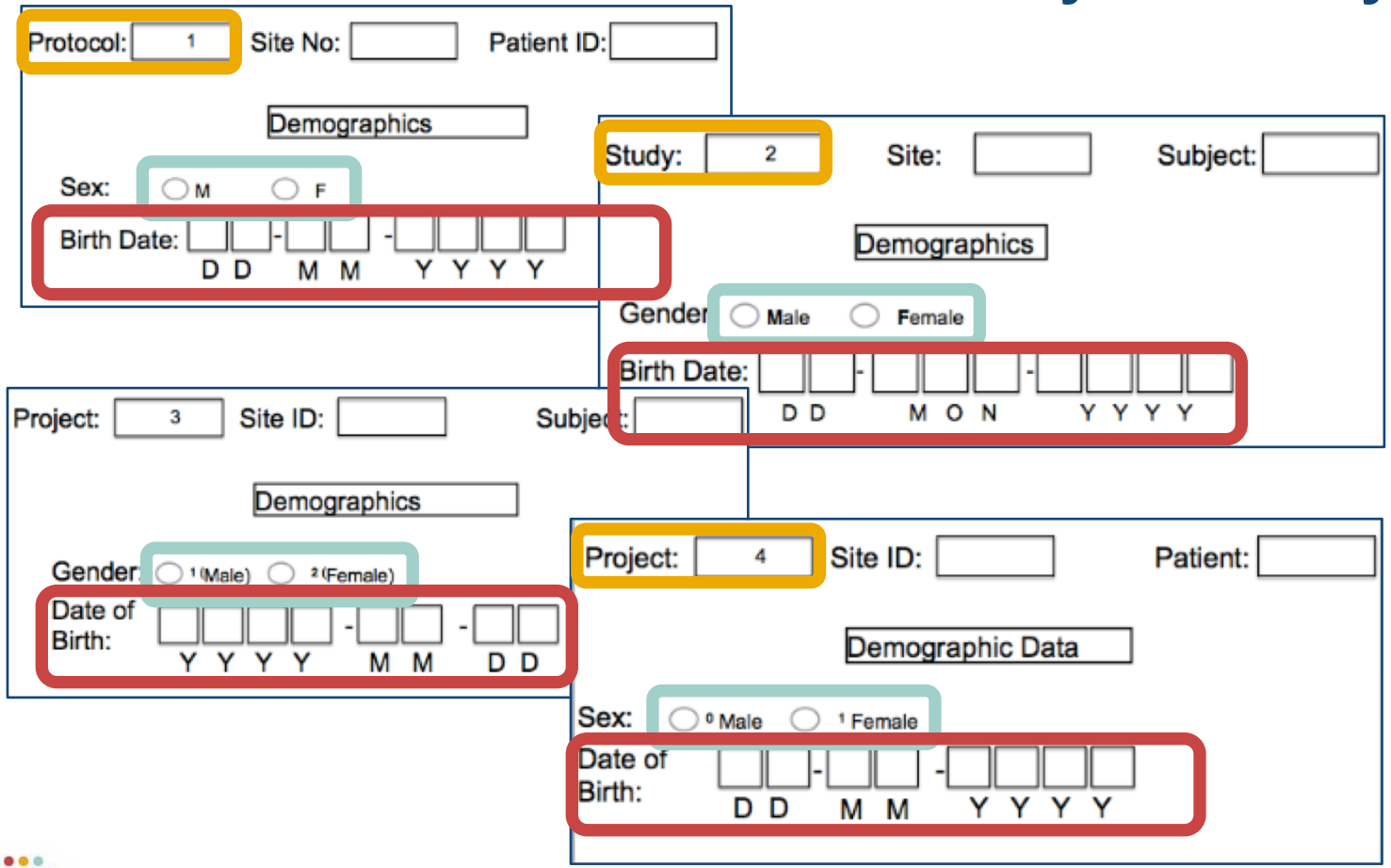
Controlled Terminology

Define-XML

TAUGs

Data Transport Standards

Unnecessary Variability…



Name for Subject ID is never the same

Study #1 – demog.xpt

SUBJID	SEX
0001	M
0002	F
0003	F
0004	M
0005	F

Study #2 – dmg.xpt

ID	GENDER
A1	Male
A2	Male
A3	Female
A4	Female
A5	Male

Name for demographics dataset varies

Gender or Sex - do these mean the same thing?

Study #3 – dmgph.xpt

PTID	GENDER
0001	1
0002	1
0003	2
0004	2
0005	1

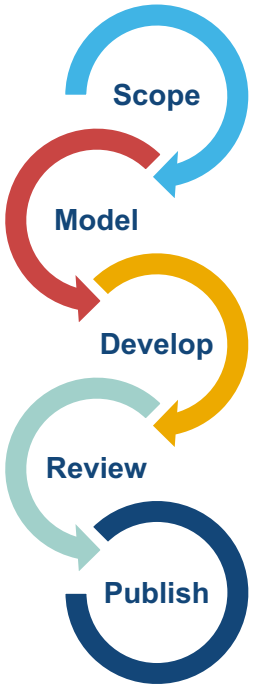
Study #4 – axd222.xpt

USUBID	SEX
00011	0
00012	1
00013	1
00014	0

Is it Male or Female, M or F, 1 or 2, or 0 or 1?

What do the numeric codes mean?

Consensus Driven Standards Development Proces



CDISC Data Standards Experts

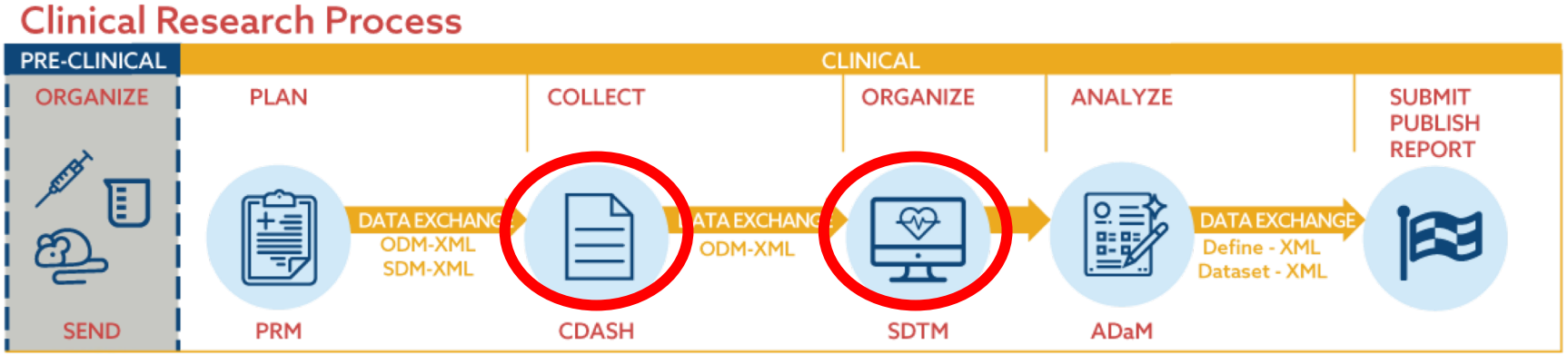
Volunteers from CDISC Member Organizations

Subject Matter Experts from Industry, Academia, Regulatory Authorities, Patient Groups, Research Consortia, Other SDOs

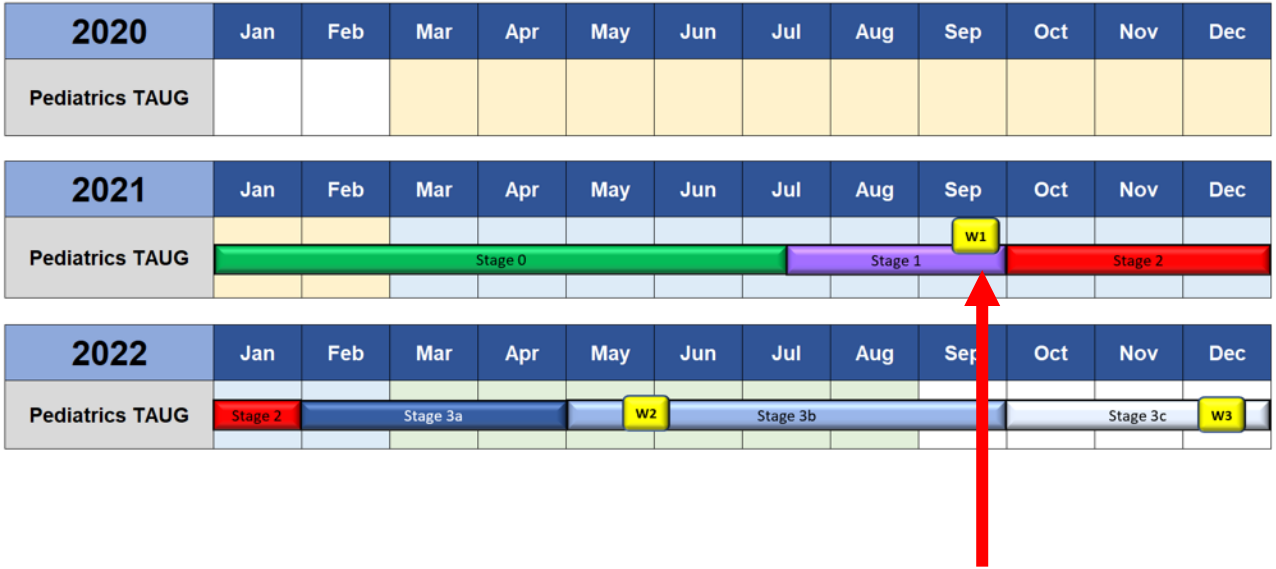
Pediatrics User Guide

- CDISC, in collaboration with IMI's Connect4Children (c4c) Project , is developing a Pediatrics User Guide

- The User Guide builds on existing CDISC standards, and will consist of data collection and data tabulation examples for use in **cross-cutting** pediatric clinical trials.



Timelines



Stage 0	Scoping and Planning
Stage 1	Identification/Modeling of Concepts
Stage 2	Standards Development
Stage 3a	Internal Review
Stage 3b	Public Review
Stage 3c	Publication
W	Public Webinars 1 - Scoping Results 2 - Public Review 3 - Publication

TAUG Deliverable Feb 2023 (M58)
Submission required April 2023 (M60)

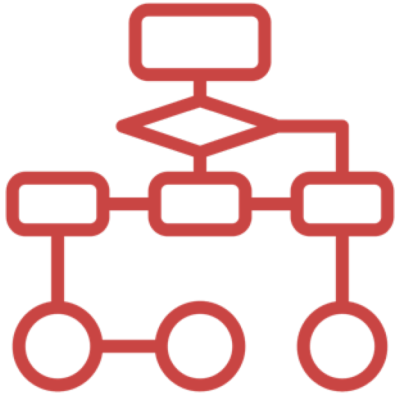
Scoping

- Ensure that the project is well defined with clear and achievable deliverables
- Perform background research to develop the initial scope

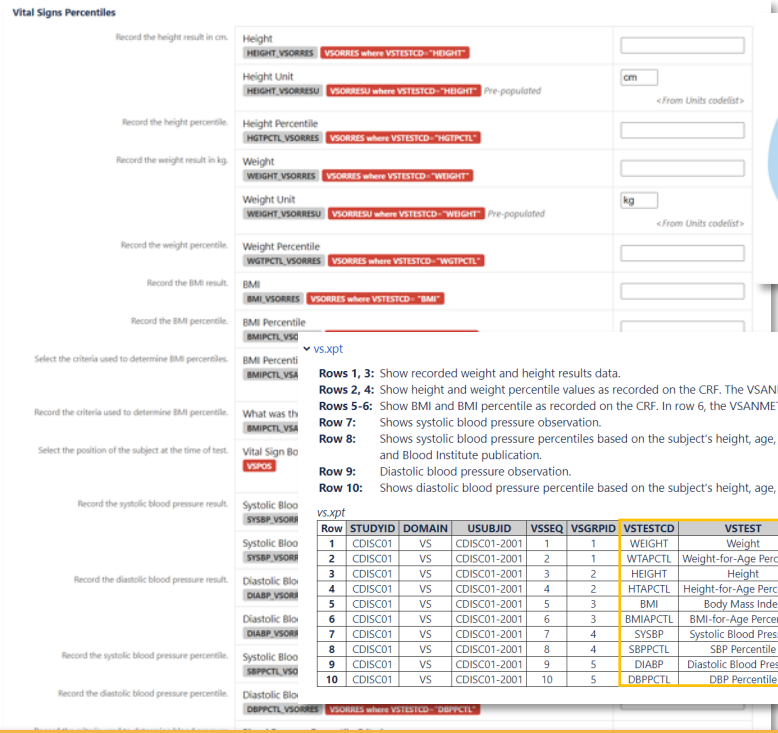
Concept Modeling

Illustrate in more detail the information that will be included in the proposed standard

- Develop concept maps to aid in semantic understanding
- Develop terminology and questionnaires, ratings and scales



Standards Development



Data Collection

CDASH

Data Tabulation

SDTM

vs.xpt

Rows 1, 3: Show recorded weight and height results data.
Rows 2, 4: Show height and weight percentile values as recorded on the CRF. The VSANMETH value "CDC Weight for Age Percentiles" indicates that the height percentile was calculated using CDC criteria.
Rows 5-6: Show BMI and BMI percentile as recorded on the CRF. In row 6, the VSANMETH value "CDC BMI Percentiles" indicates that the BMI percentile was calculated using CDC criteria.
Row 7: Shows systolic blood pressure observation.
Row 8: Shows systolic blood pressure percentiles based on the subject's height, age, sex, and systolic blood pressure. The VSANMETH value is "NHLBI Blood Pressure Percentiles", indicating that the percentile was calculated based on a National Heart, Lung, and Blood Institute publication.
Row 9: Diastolic blood pressure observation.
Row 10: Shows diastolic blood pressure percentile based on the subject's height, age, sex, and diastolic blood pressure. The VSANMETH value is "NHLBI Blood Pressure Percentiles", indicating that the percentile was calculated based on a NHLBI publication.

vs.xpt

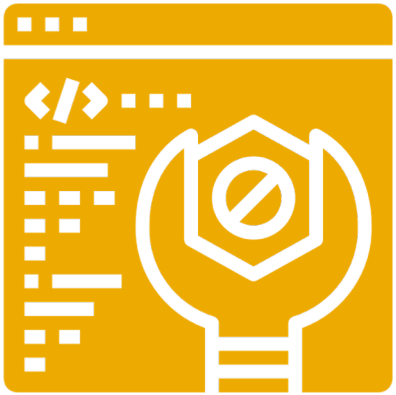
Row	STUDYID	DOMAIN	USUBJID	VSSEQ	VSGRPID	VSTESTCD	VSTEST	VSPOS	VSORRES	VSORRESU	VSSTRESC	VSSTRESN	VSSTRESU	VSRESCAT	VSANMETH	VSLOBXFL	VISITNUM	VSDTC
1	CDISC01	VS	CDISC01-2001	1	1	WEIGHT	Weight		62	kg	62	62	kg			Y	1	2018-12-21
2	CDISC01	VS	CDISC01-2001	2	1	WTAPCTL	Weight-for-Age Percentile		85		85	85			CDC Weight for Age Percentiles	Y	1	2018-12-21
3	CDISC01	VS	CDISC01-2001	3	2	HEIGHT	Height		152	cm	1.52	1.52	m			Y	1	2018-12-21
4	CDISC01	VS	CDISC01-2001	4	2	HTAPCTL	Height-for-Age Percentile		50		50	50			CDC Height for Age Percentiles	Y	1	2018-12-21
5	CDISC01	VS	CDISC01-2001	5	3	BMI	Body Mass Index		26.8	kg/m2	26.8	26.8	kg/m2			Y	1	2018-12-21
6	CDISC01	VS	CDISC01-2001	6	3	BMIAPCTL	BMI-for-Age Percentile		94		94	94		OBESE	CDC BMI Percentiles	Y	1	2018-12-21
7	CDISC01	VS	CDISC01-2001	7	4	SYSBP	Systolic Blood Pressure	SITTING	105	mmHg	105	105	mmHg			Y	1	2018-12-21
8	CDISC01	VS	CDISC01-2001	8	4	SBPPCTL	SBP Percentile		50		50	50			NHLBI Blood Pressure Percentiles	Y	1	2018-12-21
9	CDISC01	VS	CDISC01-2001	9	5	DIABP	Diastolic Blood Pressure	SITTING	63	mmHg	63	63	mmHg			Y	1	2018-12-21
10	CDISC01	VS	CDISC01-2001	10	5	DBPPCTL	DBP Percentile		50		50	50			NHLBI Blood Pressure Percentiles	Y	1	2018-12-21

Controlled Terminology

Internal and Public Reviews

- The internal review ensures that all CDISC teams and appropriate collaborative groups and subject matter experts have the opportunity to review the draft standard.
- During the public review, commenting is open to the public
- Both of these stages involve:
  - Releasing the draft standard for review
  - Resolving issues and updating the draft standard
  - Submitting remaining/additional terminology requests
  - Obtaining Global Governance Group (GGG) approval to proceed to the next stage

Publication and Maintenance

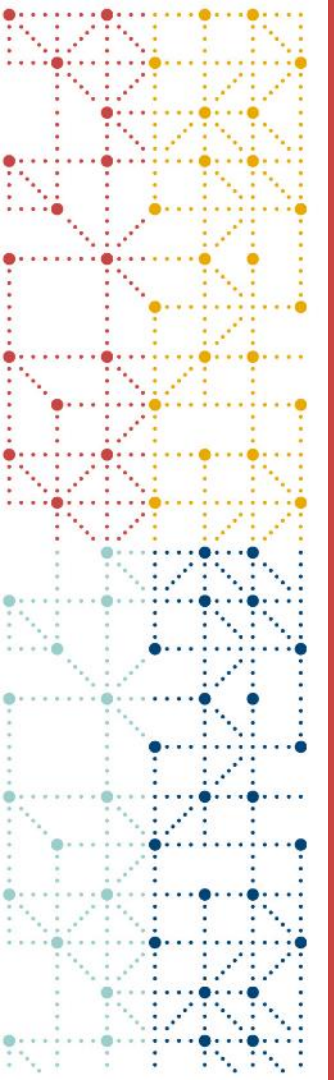


Questionnaires, Ratings, and Scales (QRS)

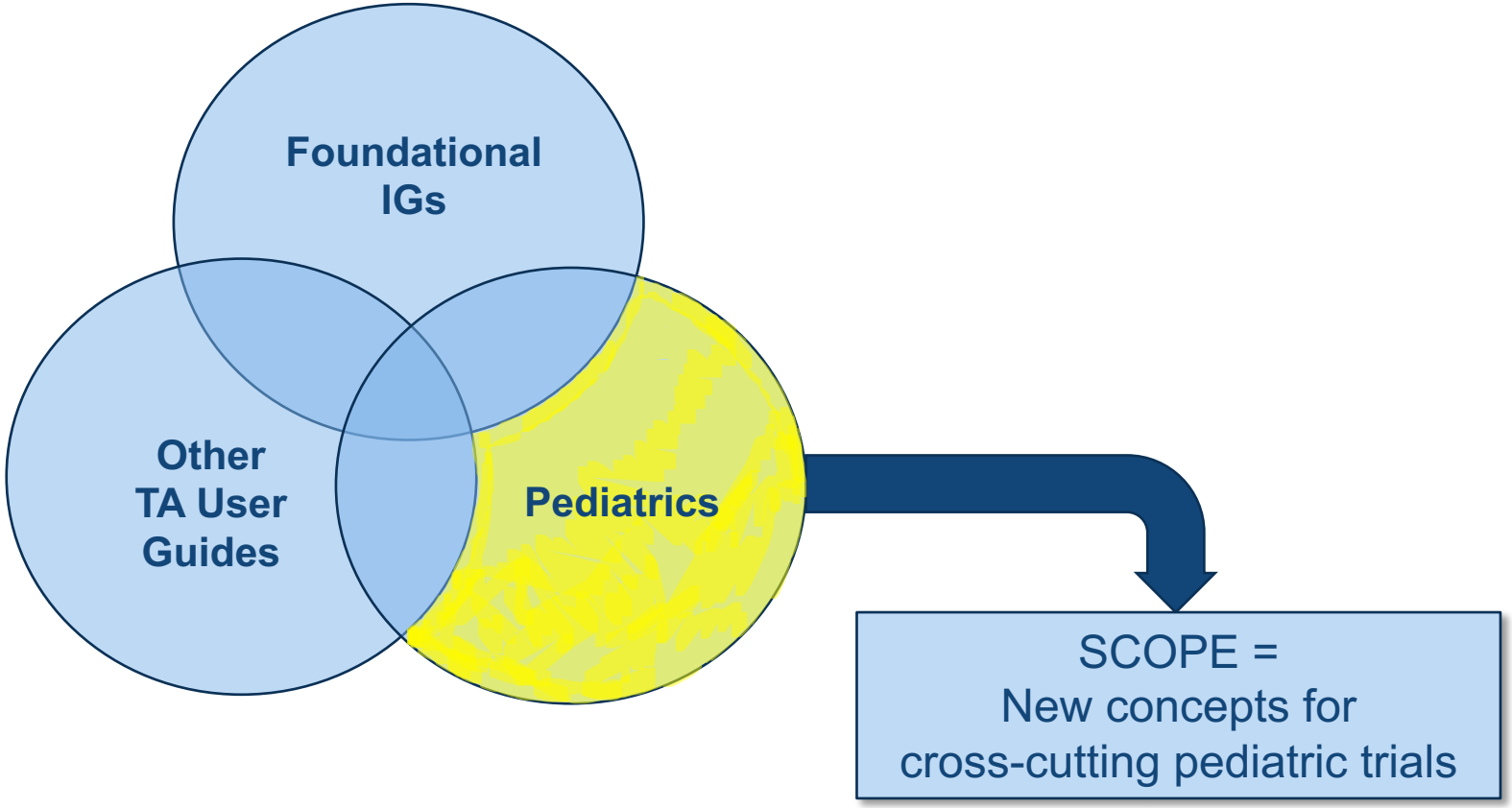
- QRS Development has its own standards development process:
  - Identification by scoping team
  - Check if QRS exists in current QRS library
  - Submit request for new QRS development
  - CDISC requests copyright permissions
  - If permission is granted, the QRS moves into development of its own supplement
  - After development, the QRS supplements pass thorough the Internal and Public Review quality gateway processed
  - Publication of the QRS supplement on the CDISC website
- Goal: Identify up to 10 standard QRS related to pediatric trials
- These will be listed as QRS of interest in the TA user guide

Terminology Development

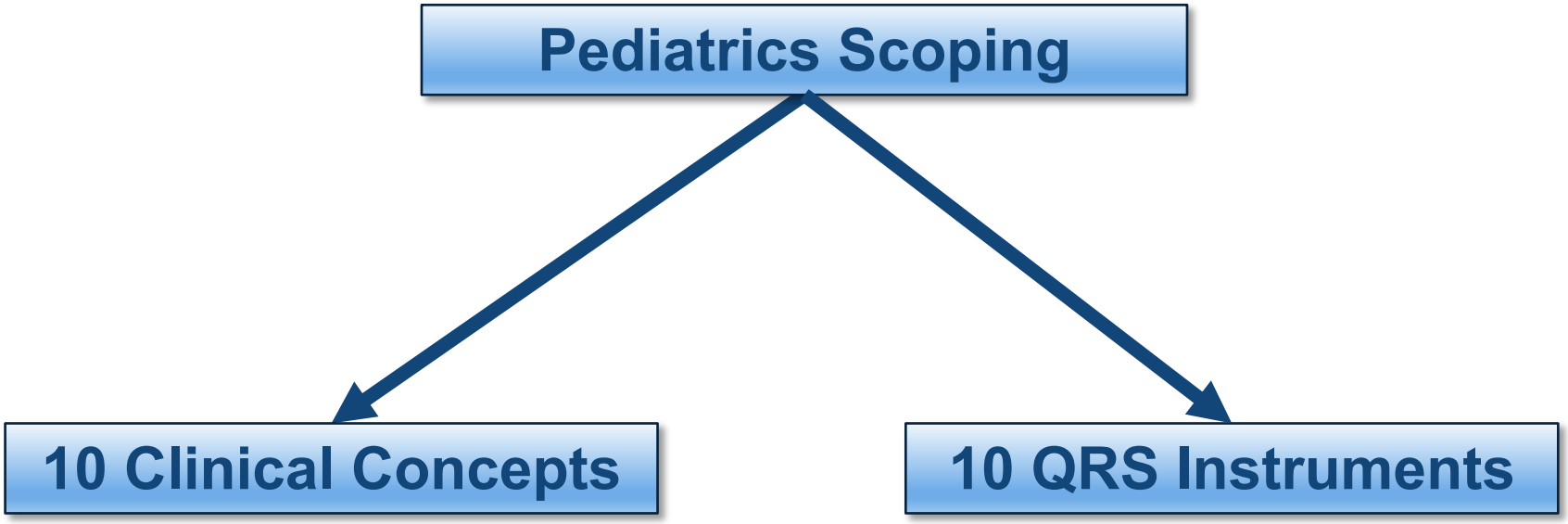
- Terminology Development has its own standards development process
- Each TA team is assigned a terminology representative from NCI-EVS
  - This individual liaises with CT teams and TA teams
- Terminology development begins during internal review
  - Relatively stable model means less re-work
  - SMEs may be needed
- Terminology analysis can inform data modeling decisions and may change as terminology development proceeds
- CDISC Terminology is published on a quarterly cycle
  - Goal is to have all terminology published or in public review by the time the TAUG is published

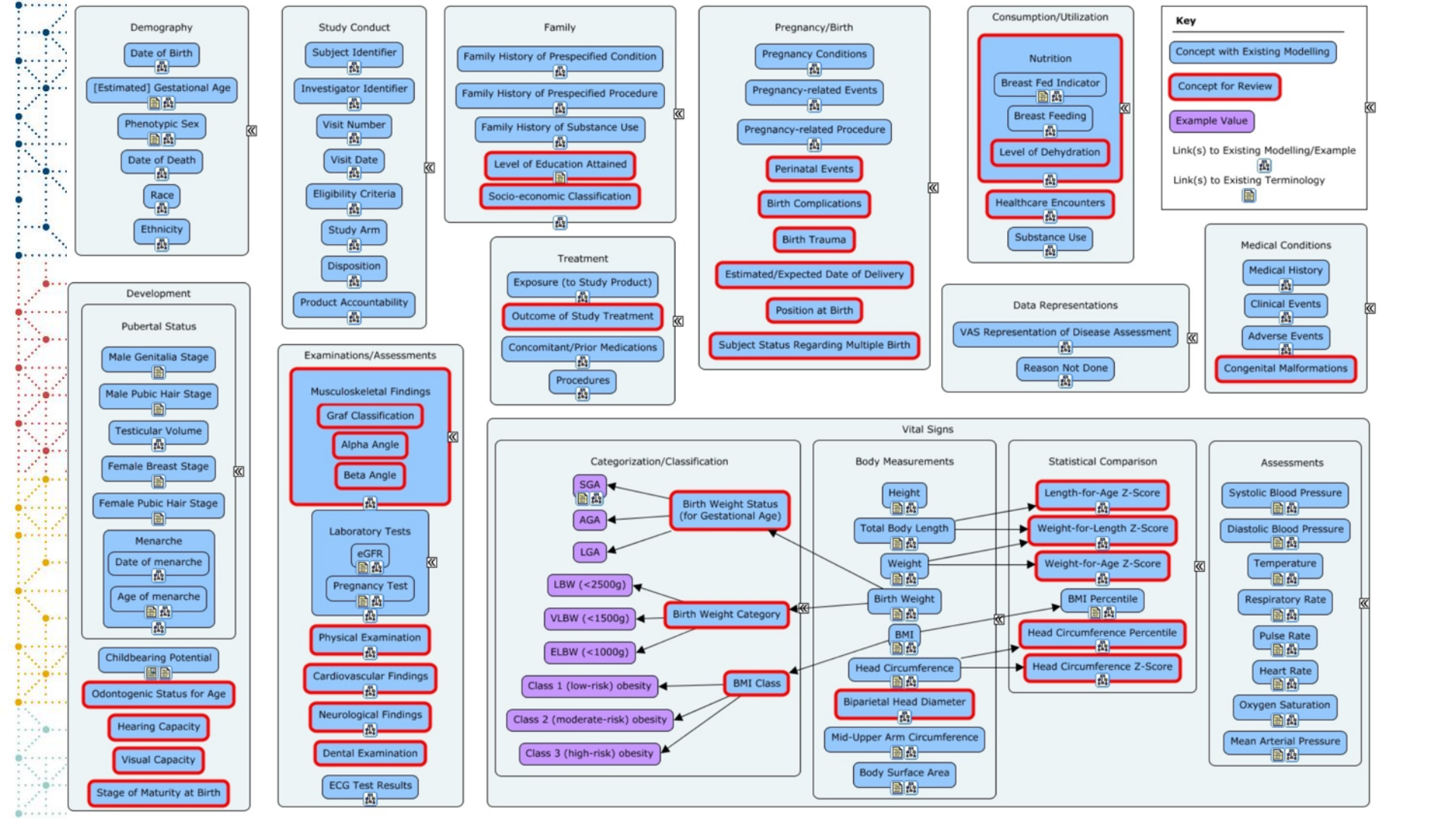


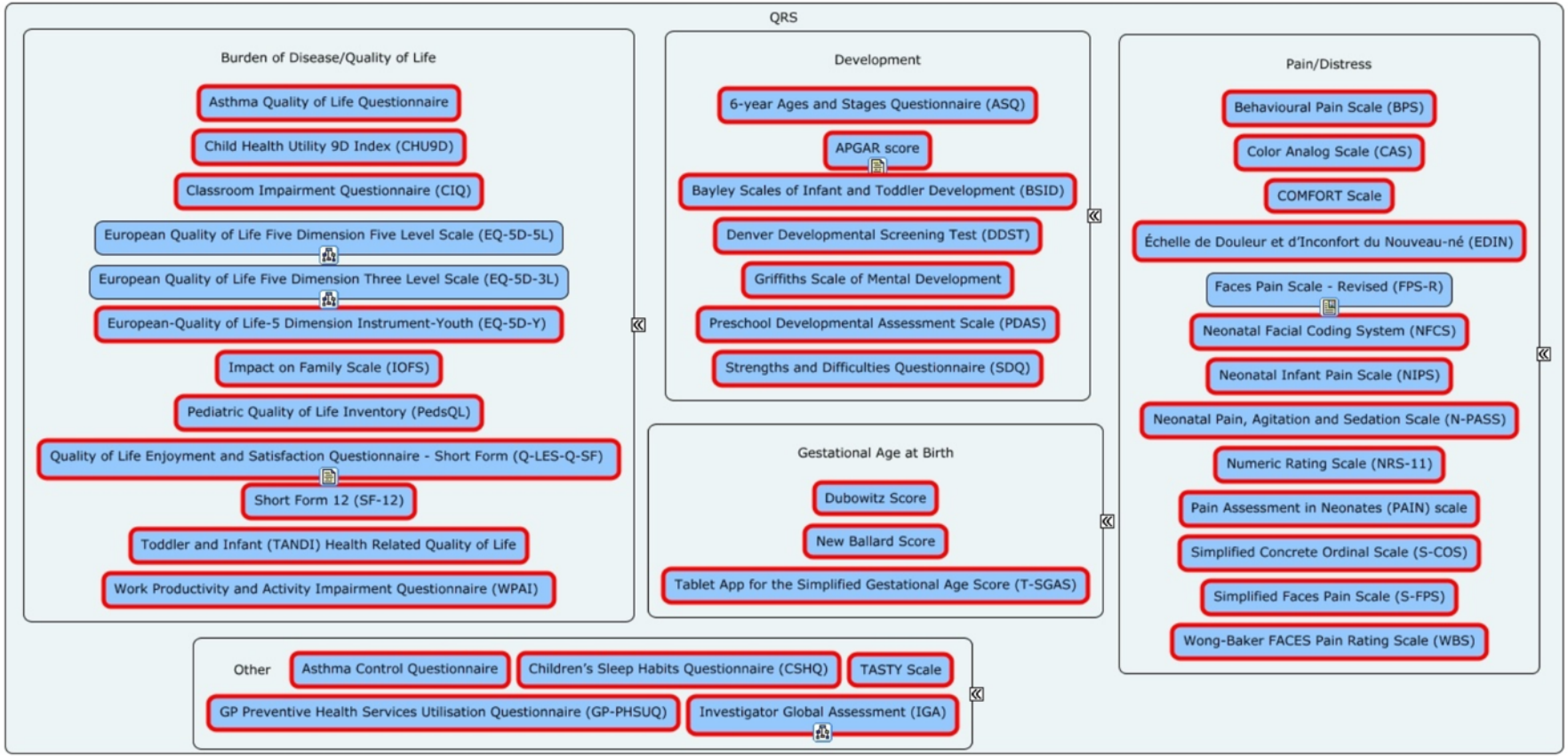
Deep Dive into Scoping


Foundational IGs
Other TA User Guides
Pediatrics
SCOPE =
New concepts for cross-cutting pediatric trials

Scoping Aims

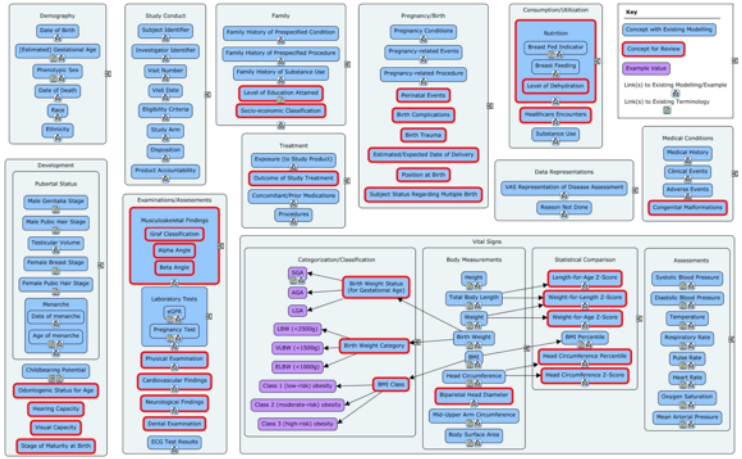



Demography
Date of Birth
[Estimated] Gestational Age
Phenotypic Sex
Date of Death
Race
Ethnicity
Development
Pubertal Status
Male Genitalia Stage
Male Pubic Hair Stage
Testicular Volume
Female Breast Stage
Female Pubic Hair Stage
Menarche
Date of menarche
Age of menarche
Childbearing Potential
Odontogenic Status for Age
Hearing Capacity
Visual Capacity
Stage of Maturity at Birth
Study Conduct
Subject Identifier
Investigator Identifier
Visit Number
Visit Date
Eligibility Criteria
Study Arm
Disposition
Product Accountability
Examinations/Assessments
Musculoskeletal Findings
Graf Classification
Alpha Angle
Beta Angle
Laboratory Tests
eGFR
Pregnancy Test
Physical Examination
Cardiovascular Findings
Neurological Findings
Dental Examination
ECG Test Results
Family
Family History of Prespecified Condition
Family History of Prespecified Procedure
Family History of Substance Use
Level of Education Attained
Socio-economic Classification
Treatment
Exposure (to Study Product)
Outcome of Study Treatment
Concomitant/Prior Medications
Procedures
Pregnancy/Birth
Pregnancy Conditions
Pregnancy-related Events
Pregnancy-related Procedure
Perinatal Events
Birth Complications
Birth Trauma
Estimated/Expected Date of Delivery
Position at Birth
Subject Status Regarding Multiple Birth
Consumption/Utilization
Nutrition
Breast Fed Indicator
Breast Feeding
Level of Dehydration
Healthcare Encounters
Substance Use
Data Representations
VAS Representation of Disease Assessment
Reason Not Done
Key
Concept with Existing Modelling
Concept for Review
Example Value
Link(s) to Existing Modelling/Example
Link(s) to Existing Terminology
Medical Conditions
Medical History
Clinical Events
Adverse Events
Congenital Malformations
Vital Signs
Categorization/Classification
SGA
AGA
LGA
Birth Weight Status (for Gestational Age)
LBW (<2500g)
VLBW (<1500g)
ELBW (<1000g)
Birth Weight Category
Class 1 (low-risk) obesity
Class 2 (moderate-risk) obesity
Class 3 (high-risk) obesity
BMI Class
Body Measurements
Height
Total Body Length
Weight
Birth Weight
BMI
Head Circumference
Biparietal Head Diameter
Mid-Upper Arm Circumference
Body Surface Area
Statistical Comparison
Length-for-Age Z-Score
Weight-for-Length Z-Score
Weight-for-Age Z-Score
BMI Percentile
Head Circumference Percentile
Head Circumference Z-Score
Assessments
Systolic Blood Pressure
Diastolic Blood Pressure
Temperature
Respiratory Rate
Pulse Rate
Heart Rate
Oxygen Saturation
Mean Arterial Pressure


QRS
Burden of Disease/Quality of Life
Asthma Quality of Life Questionnaire
Child Health Utility 9D Index (CHU9D)
Classroom Impairment Questionnaire (CIQ)
European Quality of Life Five Dimension Five Level Scale (EQ-5D-5L)
European Quality of Life Five Dimension Three Level Scale (EQ-5D-3L)
European-Quality of Life-5 Dimension Instrument-Youth (EQ-5D-Y)
Impact on Family Scale (IOFS)
Pediatric Quality of Life Inventory (PedsQL)
Quality of Life Enjoyment and Satisfaction Questionnaire - Short Form (Q-LES-Q-SF)
Short Form 12 (SF-12)
Toddler and Infant (TANDI) Health Related Quality of Life
Work Productivity and Activity Impairment Questionnaire (WPAI)
Development
6-year Ages and Stages Questionnaire (ASQ)
APGAR score
Bayley Scales of Infant and Toddler Development (BSID)
Denver Developmental Screening Test (DDST)
Griffiths Scale of Mental Development
Preschool Developmental Assessment Scale (PDAS)
Strengths and Difficulties Questionnaire (SDQ)
Gestational Age at Birth
Dubowitz Score
New Ballard Score
Tablet App for the Simplified Gestational Age Score (T-SGAS)
Pain/Distress
Behavioural Pain Scale (BPS)
Color Analog Scale (CAS)
COMFORT Scale
Échelle de Douleur et d'Inconfort du Nouveau-né (EDIN)
Faces Pain Scale - Revised (FPS-R)
Neonatal Facial Coding System (NFCS)
Neonatal Infant Pain Scale (NIPS)
Neonatal Pain, Agitation and Sedation Scale (N-PASS)
Numeric Rating Scale (NRS-11)
Pain Assessment in Neonates (PAIN) scale
Simplified Concrete Ordinal Scale (S-COS)
Simplified Faces Pain Scale (S-FPS)
Wong-Baker FACES Pain Rating Scale (WBS)
Other
Asthma Control Questionnaire
Children's Sleep Habits Questionnaire (CSHQ)
TASTY Scale
GP Preventive Health Services Utilisation Questionnaire (GP-PHSUQ)
Investigator Global Assessment (IGA)

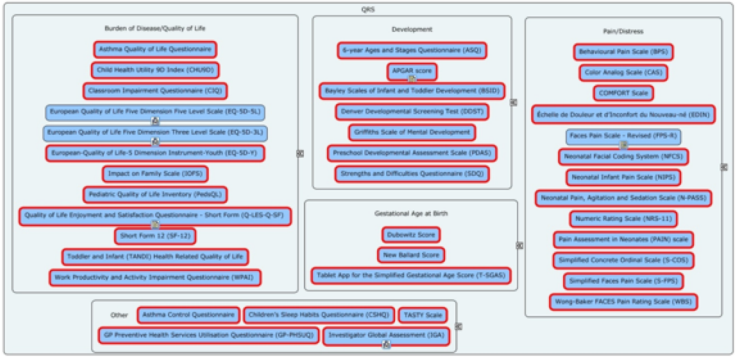
Concept Modeling

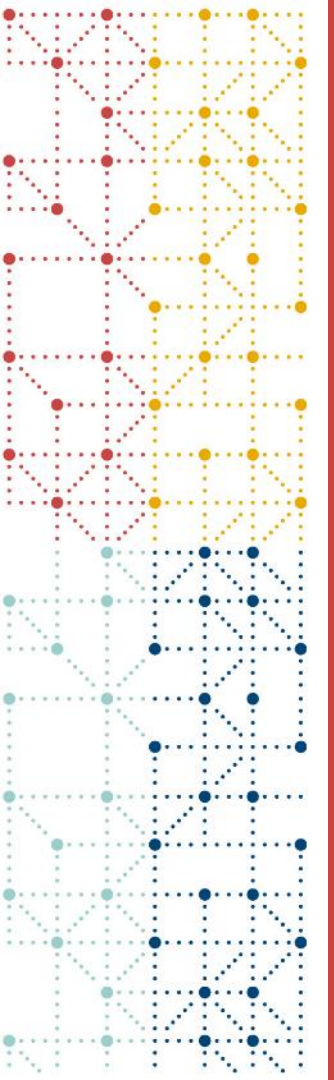
- Deep dive into areas bordered in red
- Identify where existing modelling/examples are fit for use
  - The User Guide will contain links to existing modelling/examples to avoid duplication
- Identify where new modelling/examples are needed
  - The User Guide will contain new modelling/examples
- Amend and finalize the scope based on the decisions above



QRS

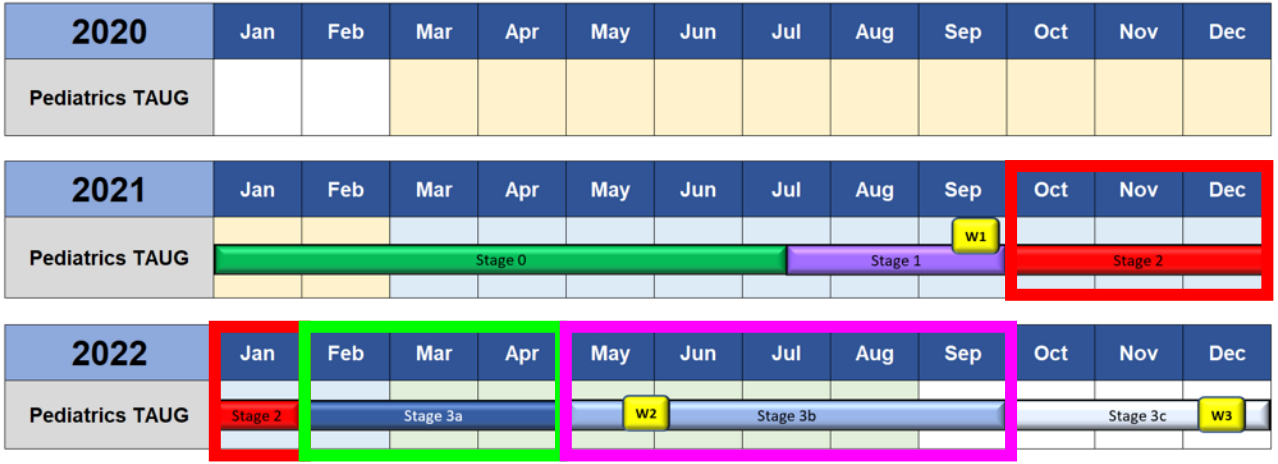
- QRS Instruments identified during scoping currently under review by the c4c expert groups in order to prioritizes the development of the most cross-cutting QRS instruments used in pediatric trials
- Once the list is prioritized, these will be pushed through to the QRS Development team
- The QRS instruments will be published as separate supplements in the CDISC website
  - https://www.cdisc.org/standards/foundational/qrs





Want to get involved?

Timelines



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W	Public Webinars 1 - Scoping Results 2 - Public Review 3 - Publication

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Submission required April 2023 (M60)

Why volunteer?

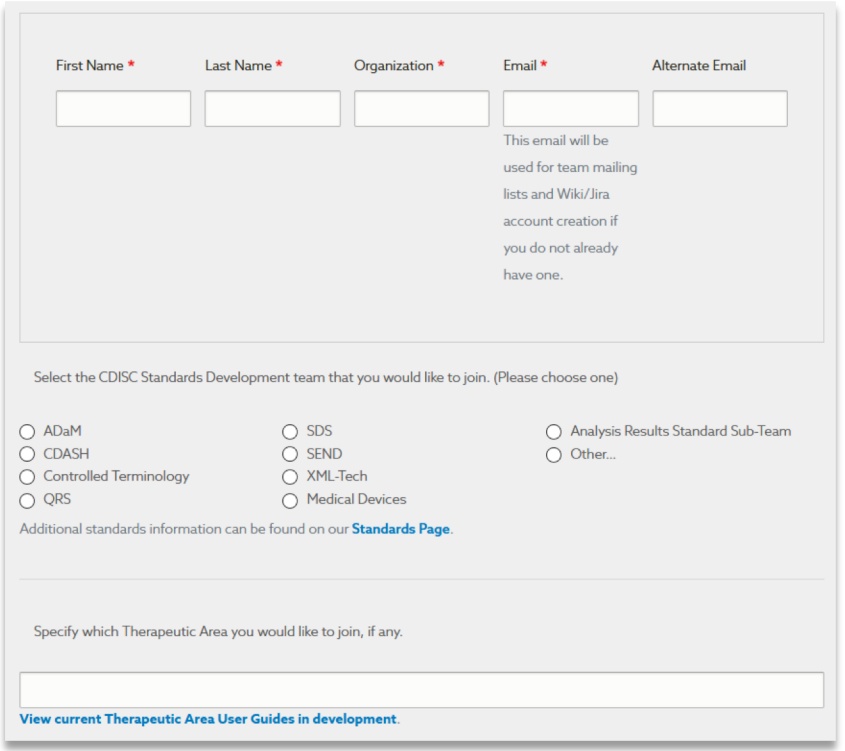
- Volunteers gain professional experience
- Teams bring people together – Networking, etc.
- Learn different things about standards and the development process
- Volunteering strengthens the standards community
- You get a chance to give back and make a difference
- Unique opportunity to influence the standard development process

cdisc

Volunteer for a Standards Development Team!

Submit an inquiry

www.cdisc.org/volunteer



First Name * | Last Name * | Organization * | Email * | Alternate Email

This email will be used for team mailing lists and Wiki/Jira account creation if you do not already have one.

Select the CDISC Standards Development team that you would like to join. (Please choose one)

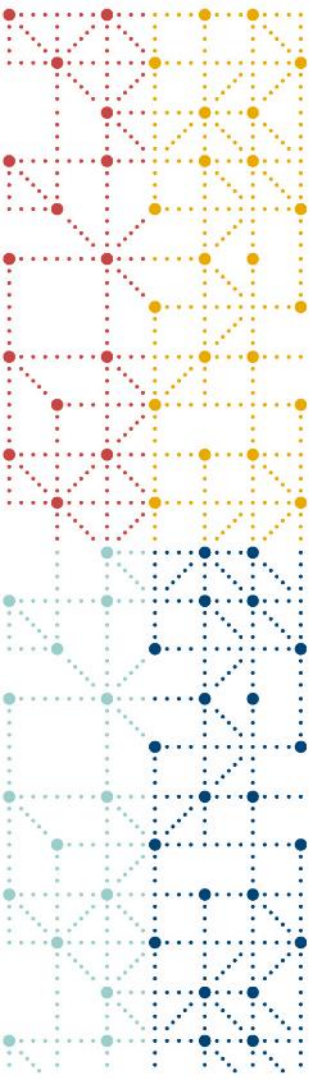
- ADaM
- CDASH
- Controlled Terminology
- QRS
- SDS
- SEND
- XML-Tech
- Medical Devices
- Analysis Results Standard Sub-Team
- Other...

Additional standards information can be found on our **Standards Page**.

Specify which Therapeutic Area you would like to join, if any.

View current Therapeutic Area User Guides in development.

Thank You!



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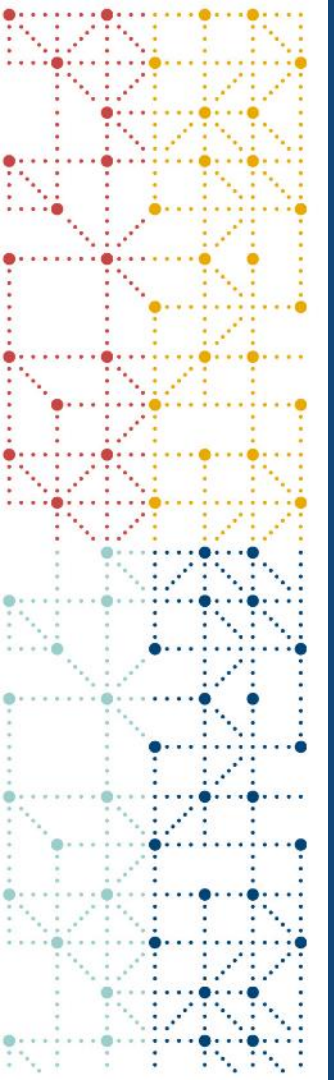

Questions & Answers

Audience Questions

In identifying QRS scales for development, will the team have a "backup list" in case some of the initial 10 do not receive copyright permission?

Audience Questions

If someone is new to CDISC standards development, can he volunteer for the standards development?

Upcoming Learning Opportunities

New Virtual Training Methods

- Information available at: www.cdisc.org
- Register at: https://learnstore.cdisc.org/
- Contact us at: training@cdisc.org

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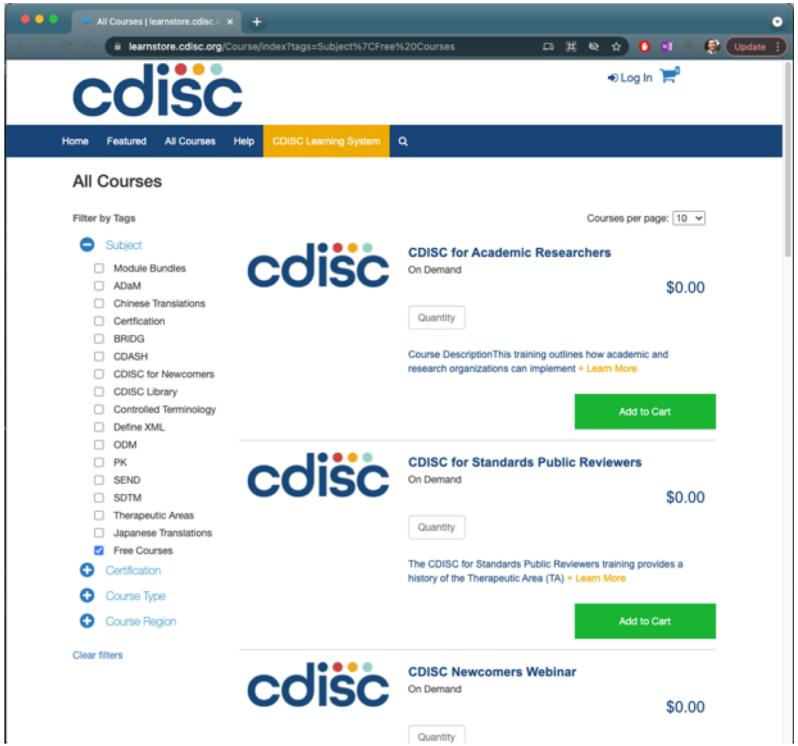
Free CDISC Courses



Http://learnstore.cdisc.org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2021 US INTERCHANGE
With Standards – Science Will Prevail!
Now fully virtual!
VIRTUAL EVENT!
Live Stream | 20-21 October
Conference & Trade Show


cdisc
2021 CHINA INTERCHANGE
With Standards – Science Will Prevail!
LIVE EVENT!
Beijing | 19-20 November
Conference | Trade Show

Upcoming Webinars

2021 Hybrid US Interchange Sneak Peek

23 September 2021, 10 - 10:45am EDT

REGISTER NOW!

Join CDISC leaders for a preview of the upcoming hybrid US Interchange , which will take place 20 - 21 OCT in person in Washington, DC and online.

CDISC staff and community experts will introduce program highlights such as the CDISC Open Rules Engine (CORE) and the CDISC Open Source Alliance (COSA), regulatory topics, and more. Webinar attendees will get a look at the conference platform that will be available for both in-person and virtual attendees. We want all our attendees to experience the same great presentations, whether from seats in the conference rooms or from the comfort of their living rooms.

Panelist(s)

Sam Hume, Vice President, Data Science, CDISC
Bernard Klinke, Virtual Experience Manager, CDISC
Amy Palmer, Head of Standards Development, CDISC
Andrea Vadakin, Sr. Director, Membership and Events, CDISC

Language

English

Controlled Terminology Updates for Q3 2021

30 September 2021, 11am - 12:30pm EDT

REGISTER NOW!

This quarterly webinar series addresses the latest Controlled Terminology release package as well as content currently in Public Review. Controlled Terminology is the set of codelists and valid values used with data items within CDISC-defined datasets. Controlled Terminology provides the values required for submission to FDA and PMDA in CDISC-compliant datasets.

Panelist(s)

Dr. Erin Muhlbradt, Clinical/Biomedical Information Specialist, Enterprise Vocabulary Services, National Cancer Institute

Language

English

Upcoming Webinars

Digital Data Flow: Project Information and Call for Volunteers

5 October 2021, 11am - 12:30pm EDT

REGISTER NOW!

CDISC, in collaboration with TransCelerate's Digital Data Flow Project, is developing a reference architecture, which will serve as a standard model for the development of a Study Definitions Repository. The Repository is a novel central component aimed at facilitating the exchange of structured study definitions across clinical systems using technical and data standards.

Deliverables will include a logical data model, supporting Controlled Terminology, API specifications and related conformance tests.

Join us as we share project progress and how to get involved.

Read the press release announcing the project.

Panelist(s)

Dave Evans, CDISC President & CEO
John Owen, CDISC Head of Partnership & Development
Christine Connolly, CDISC Senior Project Manager

Language

English

Controlled Terminology Updates for Q4 2021

21 December 2021, 11am - 12:30pm EST

REGISTER NOW!

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Panelist(s)

Dr. Erin Muhlbradt, Clinical/Biomedical Information Specialist, Enterprise Vocabulary Services, National Cancer Institute

Language

English

https://www.cdisc.org/events/webinar

Thank you!

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