

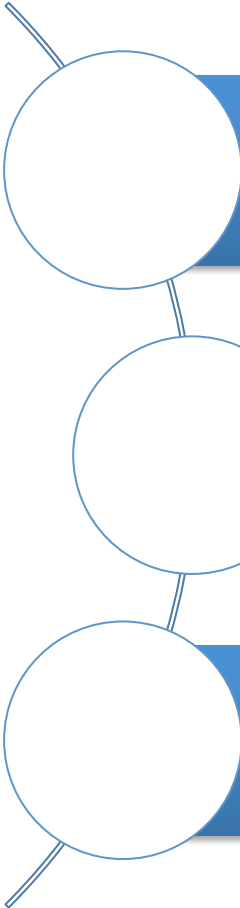
Business & Decision Life Sciences

Why don't you Define-it?

Anne-Sophie Bekx – Nick De Donder

14 October 2014



A vertical timeline graphic on the left side of the slide, consisting of three white circles connected by a thin line. The top circle has a short line extending from its top-left, and the bottom circle has a short line extending from its bottom-left.

History of Define.xml

Define-it Software Demo

Define-it versions

Introduction – History of Define.xml



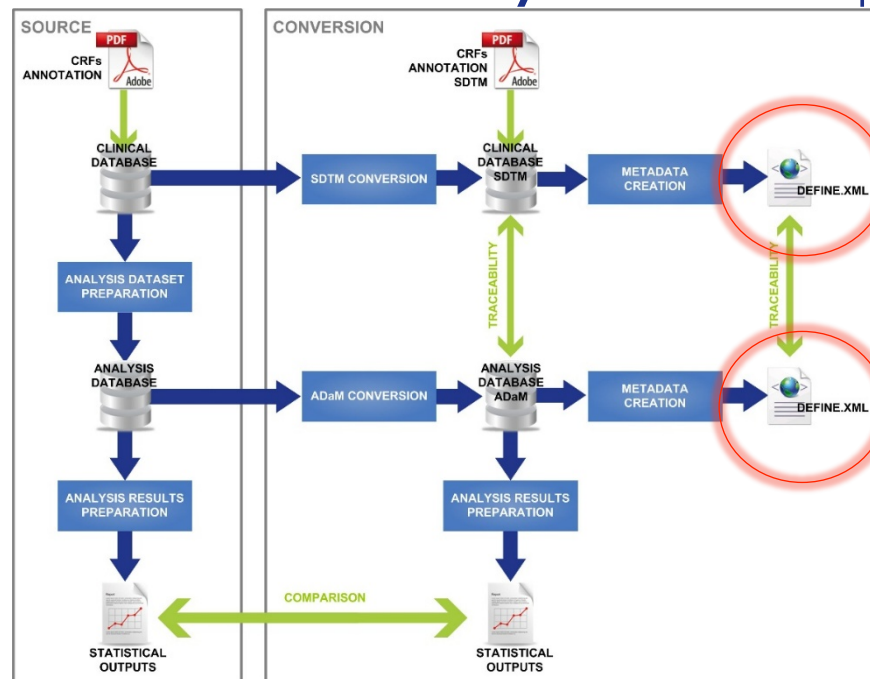
- February 2005: CRT-DDS Final Version 1.0 released for implementation (**Define.xml v1.0**)
- March 2013: **Define.xml v2.0** posted on CDISC website
 - <http://www.cdisc.org/define-xml>

A screenshot of the CDISC website. The header includes the CDISC logo with the tagline "Strength Through Collaboration" and a navigation menu with links: HOME, BLOG, SITE MAP, FAQ, CONTACT, SEARCH, PORTAL, and a Twitter icon. Below the header is a secondary navigation bar with links: ABOUT CDISC, STANDARDS & INNOVATIONS (highlighted), RESOURCES, NEWS, EDUCATION & EVENTS, MEMBERSHIP, and MEMBERS ONLY. The main content area features a section titled "Define-XML" with the subheading "Define-XML 2.0 now available for use". The text below states: "The CDISC XML Technologies team is pleased to announce the release of the Define-XML 2.0 specification. Define-XML 2.0 is a major revision of the Define-XML standard for transmission of metadata for SDTM, SEND and ADaM datasets. The model has been extensively refined in response to implementation experience with Version 1.0, evolution of the SDTM, SEND and ADaM standards and best practices by SDTM and ADaM metadata experts." Below this text, it says "Key changes to the model include:". On the right side of the main content area, there is a blue sidebar with a "Become a CDISC Member (Fee Based)" section and a "Member Log-in" section. The log-in section includes input fields for "user name" and a password (represented by dots), a "Log In" button, and a "Forgot my password" link.

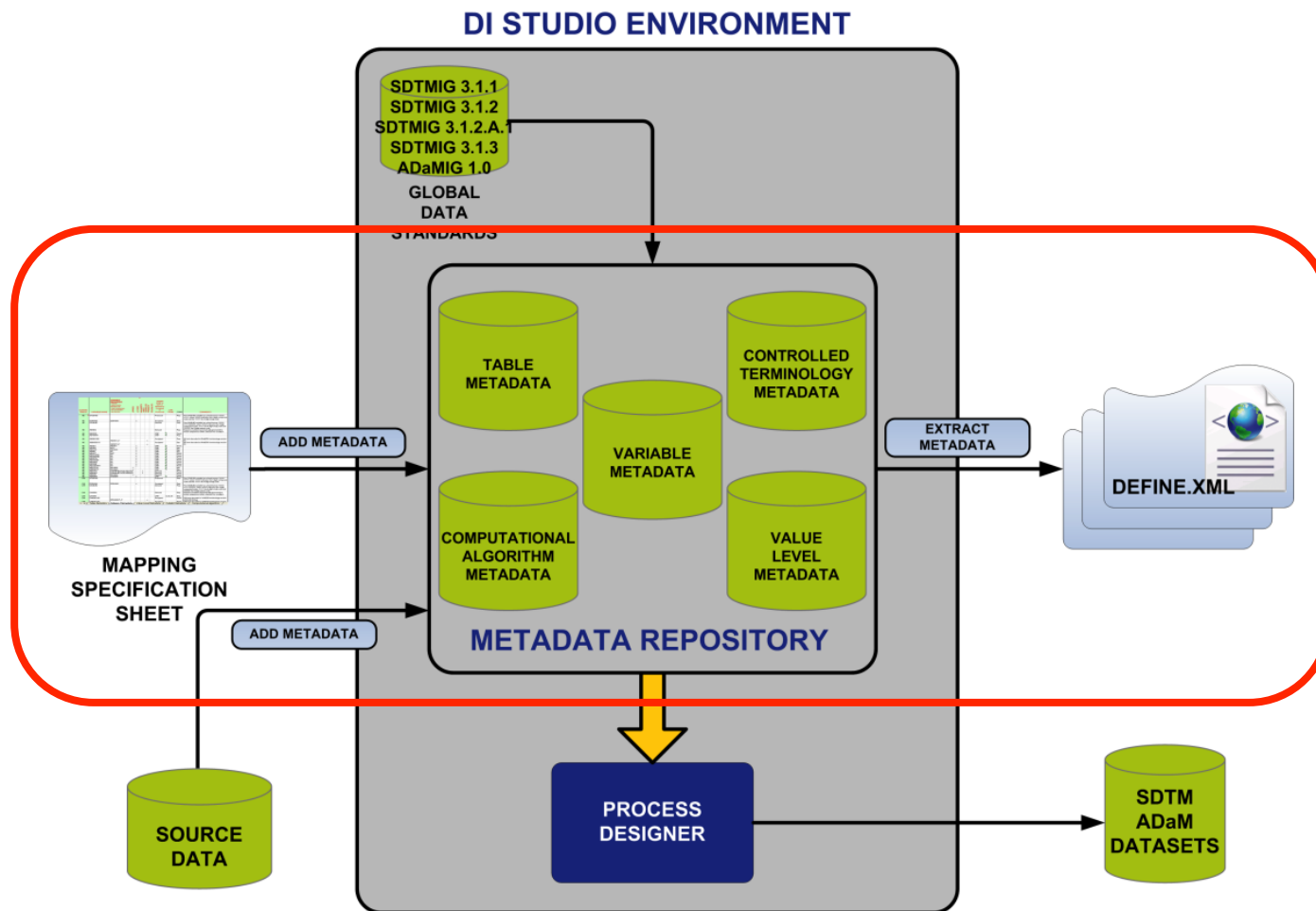
Introduction – Define.xml at BDLS



- Since 2007, BDLS has gained extensive experience in converting data for FDA submissions
- Define.xml is **a deliverable for any conversion project**:



Introduction - Define.xml at BDLS



Metadata Definitions



Table Metadata
Dataset Name
Dataset Label
Class
Structure
Purpose
Keys

Variable Metadata
Variable Name
Variable Label
Type
Controlled Terminology
Origin
Role
Comment

Value Level Metadata
Source Variable
Value
Label
Type
Controlled Terminology
Origin
Comment

Controlled Terminology
Code Value
Code Text

Computational Algorithms
Reference Name
Computation Method

Define.xml



Dataset	Description	Class	Structure	Purpose	Keys	Location
CM	Concomitant Medications	Interventions	One record per recorded medication occurrence or constant-dosing interval per subject	Tabulation	STUDYID, USUBJID, CMTRT, CMSTDTC	Tabulations\cm.xpt
EX	Exposure	Interventions	One record per constant dosing	Tabulation	STUDYID, USUBJID,	Tabulations\ex.xpt
AE	Adverse Events					

Variable	Label	Type	Controlled Terminology	Origin	Role	Comment
STUDYID	Study Identifier	text		Protocol, CRF	IDENTIFIER	The STUDYID variable has a fixed format: 'XXXX-YYYY', where 'XXXX' indicates the 4-digit compound code and the 'YYYY' the 4-digit study code

Variable	Label	Type	Controlled Terminology	Origin	Role	Comment
DOMAIN	Domain Abbreviation	text		Protocol, CRF	IDENTIFIER	
USUBJID	Unique Subject Identifier	text		Protocol, CRF	IDENTIFIER	
Value Level Metadata (ValueList.VS.VSTESTCD)						
Source Variable	Value	Label	Type	Controlled Terminology	Origin	Comment
VSTESTCD	BMI	BODY MASS INDEX	integer		Derived	See Computational Method: VS.BMI
VSTESTCD	DIABP	DIASTOLIC BLOOD PRESSURE	integer		CRF	
VSTESTCD	HEIGHT	HEIGHT	integer		CRF	
VSTESTCD	PULSE	PULSE	integer		CRF	
VSTESTCD	SYSBP	SYSTOLIC BLOOD PRESSURE	integer		CRF	
VSTESTCD	WEIGHT	WEIGHT	integer		CRF	

POSITION, Reference Name (Codelist.POSITION)	
Code Value	Code Text
STANDING	STANDING
SUPINE	SUPINE

SEX, Reference Name (Codelist.SEX)	
Code Value	Code Text
F	Female
M	Male
U	Unknown

Computational Algorithms (VS.BMI)	
Reference Name	Computation Method
VS.BMI	Equals to WEIGHT DIVIDED BY HEIGHT SQUARED
Computational Algorithms (VS.VSDY)	
Reference Name	Computation Method
VS.VSDY	Equals to (VS.VSDTC - DM.RFSTDTC) + 1 IF VSDTC is on or after RFSTDTC Equals to (VS.VSDTC - DM.RFSTDTC) IF VSDTC precedes RFSTDTC

Define.xml



- **Define.xml** has greater potential than to be used for submissions only



Define.xml v2.0



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Define-XML

[Click here to view the Team Charter.](#)

Define-XML 2.0 now available for use

The CDISC XML Technologies team is pleased to announce the release of the Define-XML 2.0 specification. Define-XML 2.0 is a major revision of the Define-XML standard for transmission of metadata for SDTM, SEND and ADaM datasets. This revision was developed in response to implementation experience with Version 1.0, evolution of the SDTM, SEND and ADaM standards and best practices by SDTM, SEND and ADaM users.

A major revision of the Define-XML standard transmission of metadata for SDTM, SEND and ADaM datasets.

Key changes to the model include:

- Support for CDISC Controlled Terminology
- Flexible definition of Value Level metadata
- Enhanced documentation of data origin
- Improved handling of comments.

Please read the PDF document [ReadMe](#) first. This document provides information about the organization of the release package and other important notes.

[Use this link to download the Define-XML 2.0 release package.](#)

Use this link to view the XML Technologies team responses to comments received during the draft comment period: [Public comment dispositions](#)

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Member Log-in
user name:
password:
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Team Charter

Major Changes



- **Documentation**
 - SDTM and ADaM covered by examples
- The way in which we handle **Value Level Metadata**
 - Value Level Metadata can be attached to any variable
- **XML Namespace**
 - Now aligned with ODM 1.3.1
- **Comments**
 - can be added on dataset level
 - are referenced
 - can be re-used
- Better support for **split domains**

Study Data Standards for Regulatory Submissions Position Statement

FDA recognizes the investment made by sponsors over the past decade to develop the expertise and infrastructure to utilize Clinical Data Interchange Standards Consortium (CDISC)[1] standards for study data. The submission of standardized study data enhances a reviewer's ability to more fully understand and characterize the efficacy and safety of a medical product.

The Prescription Drug User Fee Act (PDUFA V)[2] Performance Goals state that FDA will develop guidance for industry on the use of CDISC data standards for the electronic submission of study data in applications. In the near future, FDA will publish guidance that will require study data in conformance to CDISC standards.[3]

FDA envisions a semantically interoperable and sustainable submission environment that serves both regulated clinical research and health care. To this end, FDA will continue to research and evaluate, with its stakeholders, potential new approaches to current and emerging data standards. FDA does not foresee the replacement of CDISC standards for study data and will not implement new approaches without public input on the cost and utility of those approaches.

September 13, 2013

For more information: <http://www.fda.gov/ForIndustry/DataStandards/StudyDataStandards/default.htm>

Define.xml v2.0



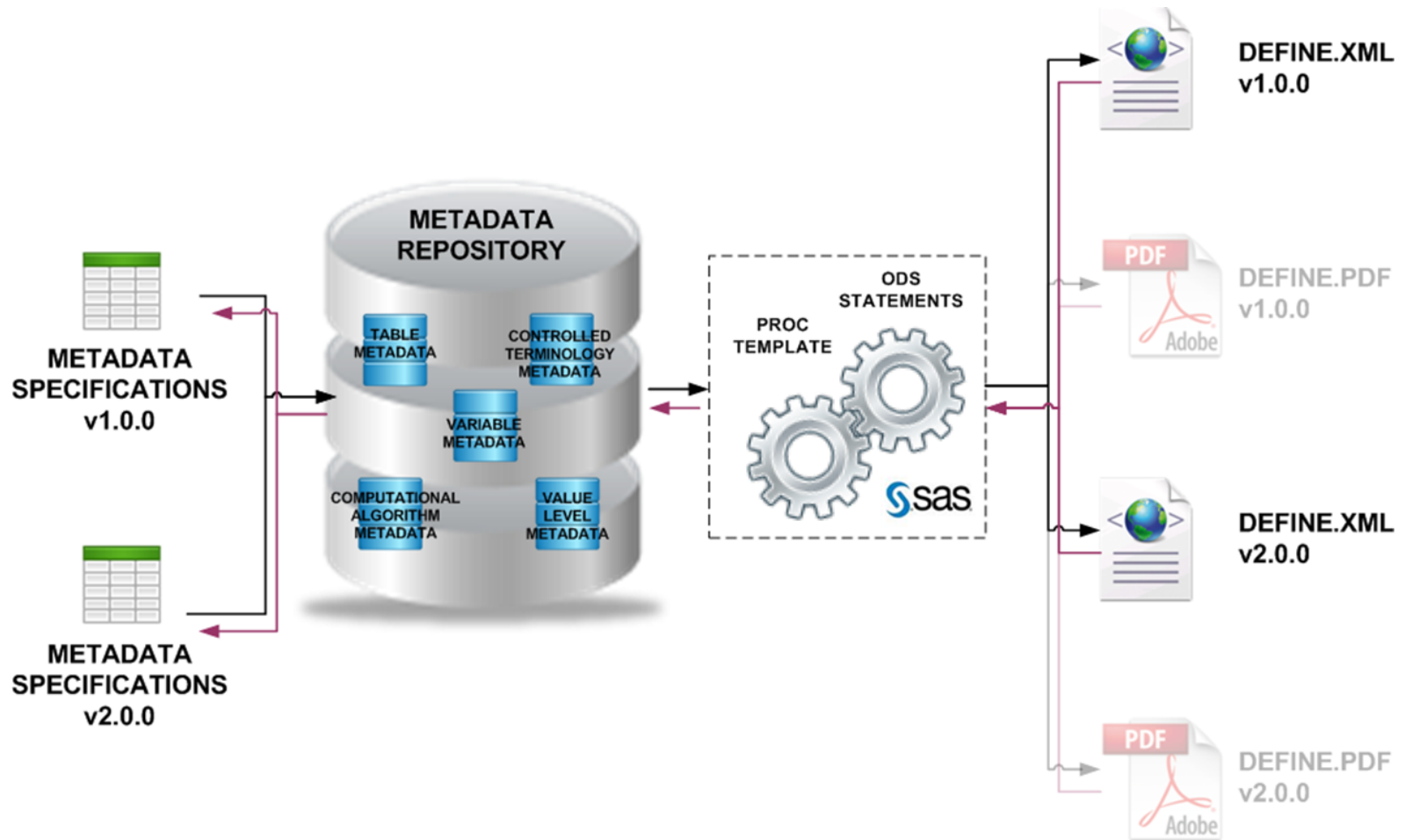
- FDA Study Data Standards Catalogue v3.0 effective 2014-01-17

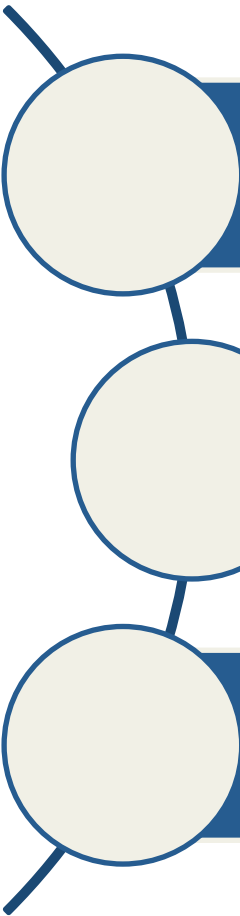
FDA Data Standards Catalog (v3.0 20140117)												
This table contains a listing of the data standards supported by FDA. The submission of standardized data using any standard not listed, or to an FDA component not listed, should be discussed with the Agency in advance.												
The standard terminology code sets are listed in a separate tab of this worksheet. Please look at the "Terminology" tab to find standard terminology information.												
	Use	Standard	Exchange Format	Standards Development Organization	Supported Version	Implementation Guide Reference	FDA Center	Date Support Begins (yyyy-mm-dd)	Date Support Ends (yyyy-mm-dd)	Date Requirements Begins	Date Requirements Ends	Regulatory References
	Clinical & Non-Clinical Study Datasets											
	Clinical study datasets	SDTM	XPT	CDISC	1.3	3.1.3	CDER, CDER	2012-12-01				http://www.cdisc.org/sdtm
	Clinical study datasets	SDTM	XPT	CDISC	1.2	3.1.2 Amendment 1	CDER, CDER	2013-08-07				http://www.cdisc.org/sdtm
	Clinical study datasets	SDTM	XPT	CDISC	1.2	3.1.2	CDER, CDER	2009-10-30				http://www.cdisc.org/sdtm
	Clinical study datasets	SDTM	XPT	CDISC	1.1	3.1.1	CDER, CDER	Ongoing	2015-01-28			http://www.cdisc.org/sdtm
	Clinical study datasets	ADaM	XPT	CDISC	2.1	1.0	CDER, CDER	Ongoing				http://www.cdisc.org/adam
	Animal study datasets	SEND	XPT	CDISC	1.2	3.1.2	CDER	2011-06-13				http://www.cdisc.org/send
	Clinical study data definition	Define	XML	CDISC	2.0	N/A	CDER, CDER, CDRH	2013-08-07				http://www.cdisc.org/define-xml
	Clinical study data definition	Define	XML	CDISC	1.0	N/A	CDER, CDER, CDRH	Ongoing				http://www.cdisc.org/define-xml

THE FDA IS NOW ACCEPTING DEFINE.XML V2.0!

- **Translates** metadata specifications into Define 1.0 and Define 2.0
- **Reads** legacy Define.xml files into metadata specifications
- **Main features:**
 - **Generates** Define.xml v1 and v2 from metadata specifications
 - **Converts** Define.xml v2 to Define.xml v1
 - **Converts** Define.xml v1 and v2 into metadata specifications

Define-it



A vertical navigation bar on the left side of the slide, consisting of three light beige circles connected by a dark blue line. The top circle is slightly offset to the left.

History of Define.xml

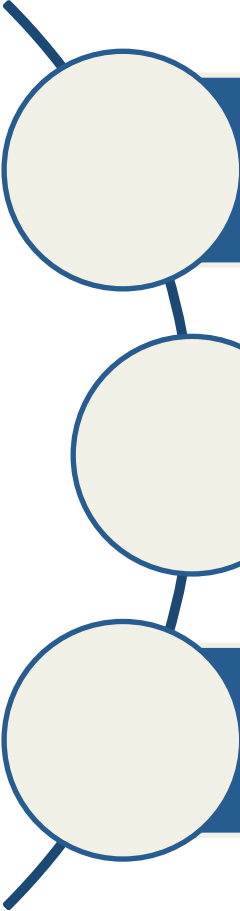
Define-it Software Demo

Define-it versions

- Key features:

- **Generate** Define.xml v1 and v2 from metadata specifications
- **Convert** Define.xml v2 to Define.xml v1
- **Convert** Define.xml v1 and v2 into metadata specifications

- Benefits:
 - “Define-it” provides :
 - Deliverables that are **fully CDISC compliant**
 - A **proven technology**
 - the Define-It solution is SAS and XML based
 - A **user friendly** solution
 - A **highly customizable** solution

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History of Define.xml

Define-it Software Demo

Define-it versions

Define-it versions



- Stand-alone desktop version:

- Presented version is the **stand-alone desktop edition**; it will run on SAS PC
- It will not run on SAS or file servers and is intended for use by a single user

- Enterprise version:

- Q2-2014: planned release of the **Enterprise version**; it will run on SAS and file servers
- Pricing will be based on:
 - Server license (first year & renewal)
 - Number of end-users
 - Number of studies
 - Implementation & Validation Services required
 - Customizations required

Potential future functionalities



- The tool will be enhanced with **ADaM metadata**
- Define **file structure validation** functionality
- **Selection of the domains** you want to report in define.xml
- Read metadata specifications v1.0 to **pre-populate** (where possible) metadata specifications 2.0
- **Read** dataset structure to pre-fill the metadata specifications
- Pre-filling of metadata spreadsheets with published **CDISC metadata** and **Controlled Terminology**
- Reading electronic metadata from **SHARE**

Product features and roadmap



	Desktop	Enterprise
Current Software Features		
Convert SDTM Define.xml (V1.0 or V2.0) into SDTM metadata specifications	✓	✓
Convert SDTM metadata specifications into SDTM Define.xml (V1.0 or V2.0)	✓	✓
Potential Future Functionalities		
Convert SDTM metadata specifications into SDTM Define.pdf (V1.0 or V2.0)		✓
Convert ADaM Define.xml (V1.0 or V2.0) into ADaM metadata specifications	✓	✓
Convert ADaM metadata specifications into ADaM Define.xml (V1.0 or V2.0)	✓	✓
Define format validation functionality		✓
Domain subsetting		✓
Metadata extraction from SAS datasets to pre-fill specifications		✓
Specifications pre-filling with published CDISC Controlled Terminology		✓

Product features and roadmap



	Desktop	Enterprise
Deployment Options		
Desktop Client/PC SAS	✓	
SAS/File server hosting		✓
Multi-user		✓
Quality and Regulatory Compliance		
Testing by worldwide community of users	✓	✓
Validated software		✓
GxP and 21 CFR Part 11 compliance		✓
Support		
Community support forum	✓	✓
E-mail and telephone support		✓

Thank you for your attention.

Join us for a coffee at the our stand.

London, United Kingdom, 14 OCT 2014





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