Solving Unique Data Management Challenges in Rare Disease Trials

04 June 2025

Questions

Use the Chat feature in Teams at any time to ask questions or share feedback.

Recording

You'll receive a link to the webinar recording via email after the event.

Resources

Check out previous webinars and more about DFnet at https://dfnetresearch.com

Speakers

Busisiwe Mbali Mpila
Client Success Manager, DFnet
South Africa

Karolyn Scott
Training and Support Specialist, DFnet
United States

Sandra Nevills
Data Management Team Lead, DFnet
Canada

About DFnet

- Providing eClinical software solutions that help researchers and biopharmaceutical companies move science forward;
- 30+ years of evolution in clinical data management and analytics services for clinical development;
- Multimodal capture: desktop, tablet, mobile; EDC, eSource, ePRO, paper;
- Our CDMS and suite of professional services help optimize your complex clinical studies complex clinical studies including rare disease trials;
- Cornerstone for data management across research sectors, phases, therapeutic areas, and sample sizes.

MZANSI dfnet dfnet

South Africa | USA | Canada

21 CFR 11 Ready

HIPAA Business Associate Agreement Available

ICH GCP compliant

cdisc GOLD MEMBER

ISO 9001:2015 Certified

GDPR Ready

CERTIFIED ISO 27001

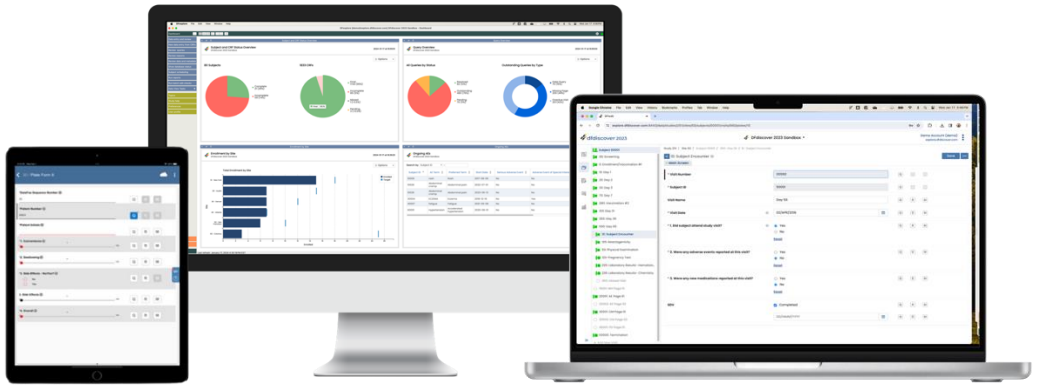
Expert Services and Powerful Tools for Better Clinical Data

Expert Data Services

- **End-to-end data management:** CDASH setup, CRF development, validation, QA, and closeout
- **Regulatory-ready documentation:** Protocol support, data management plans, and compliance deliverables
- **Clinical data insight:** SDTM/ADaM conversion, analysis plans, and reporting dashboards

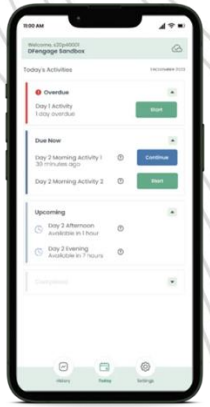
Trusted eClinical Solutions

- **Flexible data capture** with DFdiscover (EDC), DFcollect (online/offline), and DFengage (ePRO)
- **Decentralized-ready tools:** DFconsent for eConsent and DFsources for remote source review
- **Validated & 21 CFR Part 11–compliant**, with secure audit trails and seamless integration



EDC

Transcribed or direct data capture; online or offline



ePRO

Direct data capture from study participants

Data integration

with external systems via API

dfdiscover

Data import

from third party sources

Remote monitoring

Capture source data as PDF, image, audio, video

Paper CRFs

Image-based data capture

Quality Data is Mission-Critical in Rare Disease Clinical Trials

- → Over **7,000** rare diseases affect **300+ million** people globally
- → Yet **~95%** of rare diseases lack approved treatments
- → Every trial is a high-stakes opportunity to make an impact – each data point matters
- → Complex protocols, small sample sizes, and regulatory urgency require a specialized approach

- ✓ Smart data design reduces patient burden and accelerates timelines
- ✓ Clean, reliable data supports safe & effective treatments
- ✓ Timely, accurate data supports faster decision-making
- ✓ Best practices in data management lay the foundation for success

Best Practices in Clinical Data Management

Clinical Data Management Best Practices

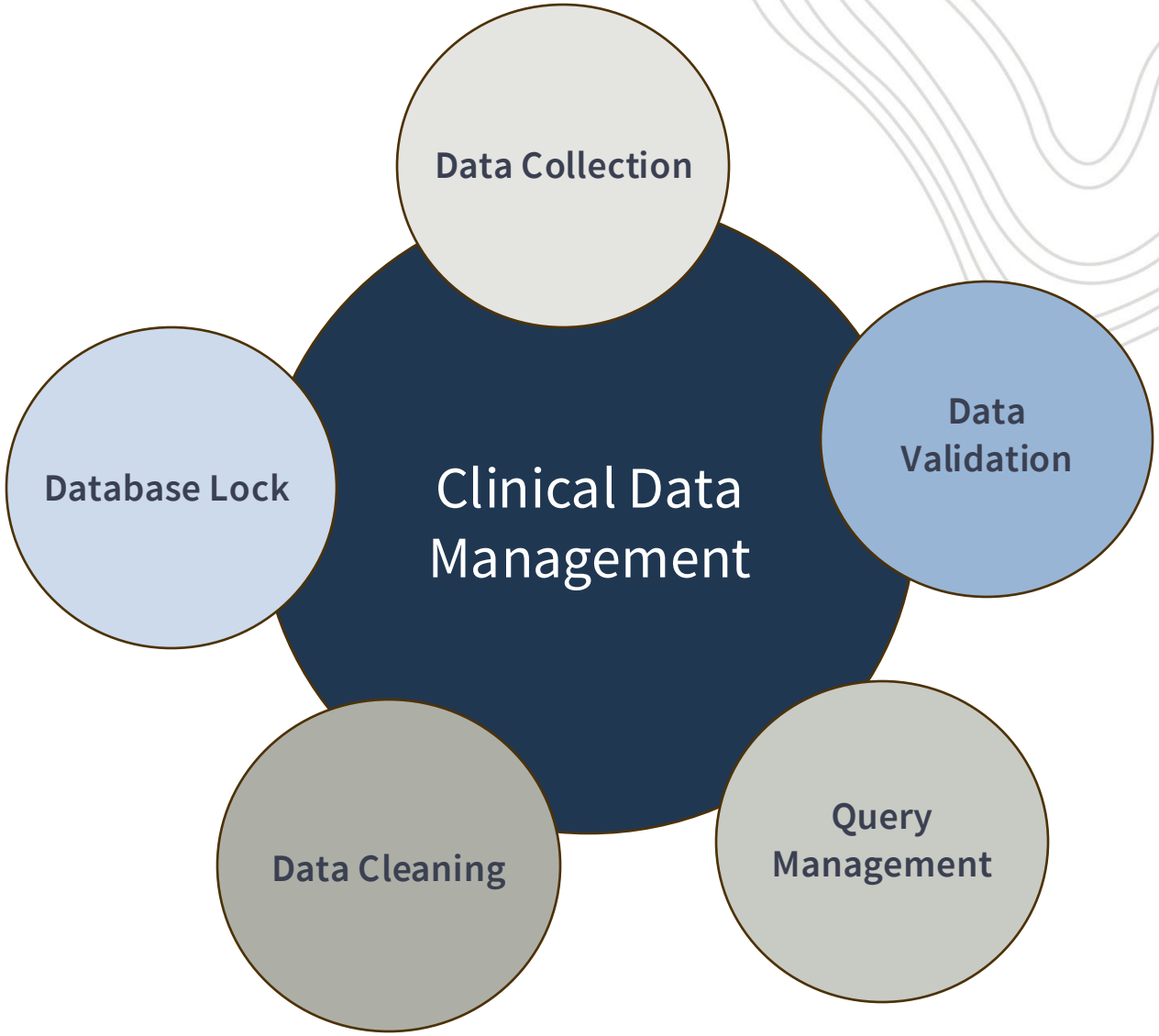
What are CDM Best Practices?

- Best practices encompass the process of collecting, cleaning and managing subject data in compliance with regulatory standards

Why do we need them?

- To produce reliable, high-quality data to support scientific conclusions

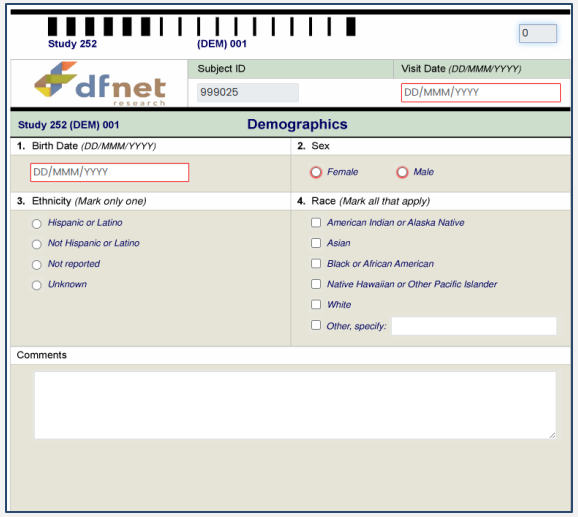
Core Components of Clinical Data Management



Consider Data Management During Protocol Design

1 Best Practice:

Plan Early


Study 252 (DEM) 001

dfnet research

Subject ID: 999025

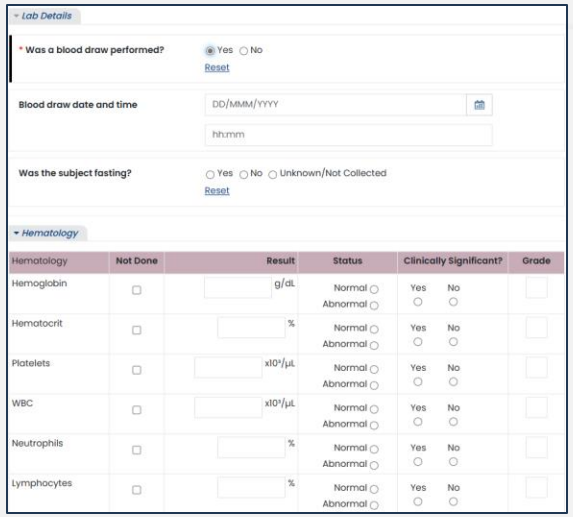
Visit Date (DD/MMM/YYYY): DD/MMM/YYYY

Study 252 (DEM) 001 Demographics

1. Birth Date (DD/MMM/YYYY): DD/MMM/YYYY
2. Sex: Female, Male
3. Ethnicity (Mark only one): Hispanic or Latino; Not Hispanic or Latino; Not reported; Unknown
4. Race (Mark all that apply): American Indian or Alaska Native; Asian; Black or African American; Native Hawaiian or Other Pacific Islander; White; Other, specify:

Comments

eCRFs/Paper/Hybrid


Lab Details

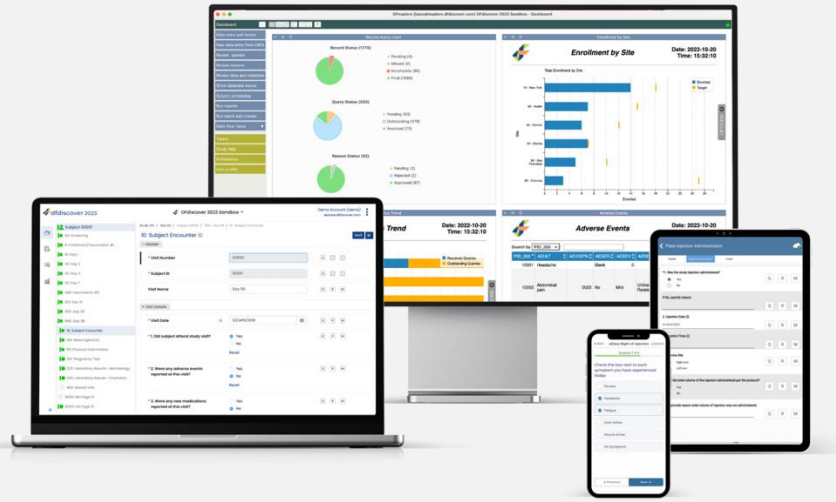
* Was a blood draw performed? Yes No Reset

Blood draw date and time: DD/MMM/YYYY hh:mm

Was the subject fasting? Yes No Unknown/Not Collected Reset

Hematology

Hematology	Not Done	Result	Status	Clinically Significant?	Grade
Hemoglobin		g/dL	Normal / Abnormal	Yes / No	
Hematocrit		%	Normal / Abnormal	Yes / No	
Platelets		x10³/µL	Normal / Abnormal	Yes / No	
WBC		x10³/µL	Normal / Abnormal	Yes / No	
Neutrophils		%	Normal / Abnormal	Yes / No	
Lymphocytes		%	Normal / Abnormal	Yes / No	



Data Collection online, offline, ePRO, etc.

1 Best Practice:

Plan Early

Involve Relevant Stakeholders

SDV Status (monitor use only)

Not selected for SDV Selected for 25% SDV Selected for 100% SDV

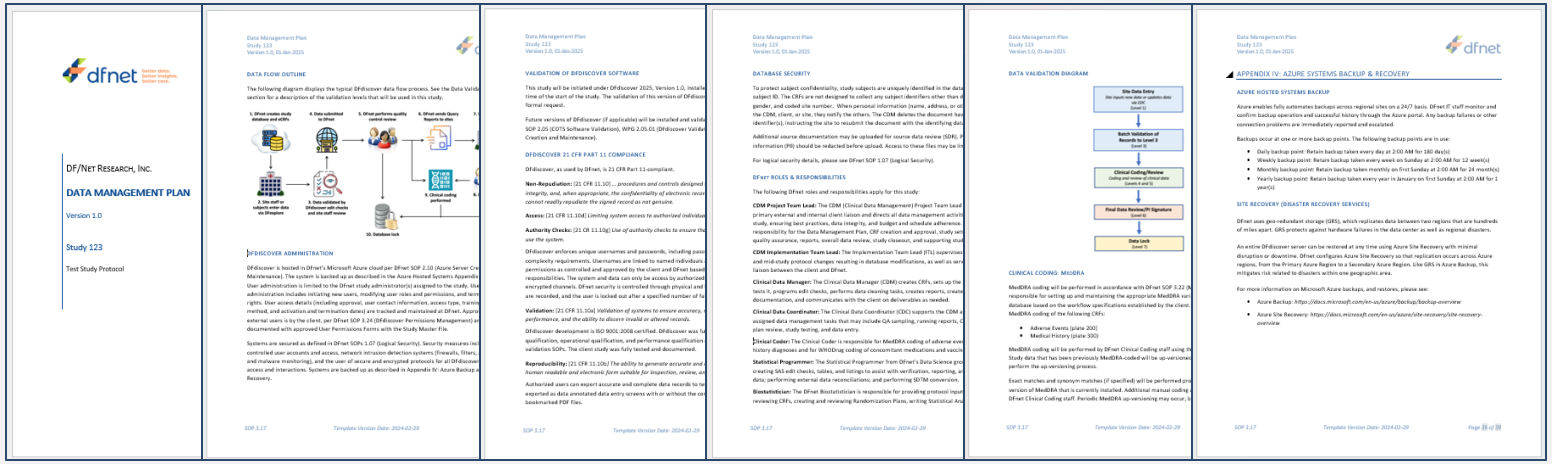
Completed by monitor: SDV Complete Data changed after SDV DD/MMM/YYYY

Monitors, Medical Reviewers, etc.

DFSTUDY	DFPLATE_DESC	SUBJID	DFVISIT_LABEL	LBDAT	LBTIM	LBORRES_n.DFVAR_DESC	ORRES_	_n.DFVA	LBORRES_n.DFVAR_DESC	ORRES_	_n.DFVA
804	Laboratory Results (eCRF)	901-ABC-001	Day 1 CLINIC	10/OCT/2023	13:24	Hemoglobin Result	14	g/dL	Hematocrit Result	25	%
804	Laboratory Results (eCRF)	901-ABC-001	Day 8 CLINIC	19/MAY/2022	15:33	Hemoglobin Result	18	g/dL	Hematocrit Result	50	%
804	Laboratory Results (eCRF)	901-ABC-002	Day 1 CLINIC	14/JUN/2023	09:25	Hemoglobin Result	16	g/dL	Hematocrit Result	35	%

Any data imports/exports?

Develop a Data Management Plan

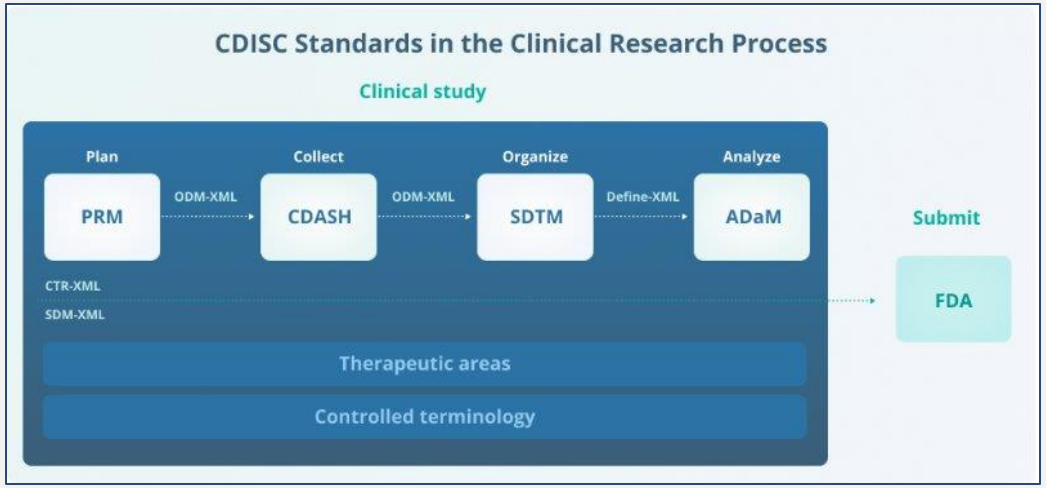


2 Best Practice:

Standards and Compliance

Implement CDISC standards

- CDASH, SDTM, ADaM



Choose a fully regulated EDC to meet ICH GCP (R3), 21 CFR Part 11, GDPR, and other critical compliance standards

- **With a built-for-purpose CDMS you…**
  - Comply with regulatory requirements
  - Ensure data integrity & security
  - Establish clear traceability & governance

3 Best Practice:

Choose Appropriate Data Capture Tools

4 Best Practice:

System Validation & Data Integrity

System Validation

Extensive Database Testing (initial and mid-study)

- Internal/external UAT
- Peer review
- Clinical Coding
- Role/database access
- Visit/conditional map

Audit Readiness

- Documentation/training up to date
- Data processes verifiable

5 Best Practice:

Training

Tailor to Each Role

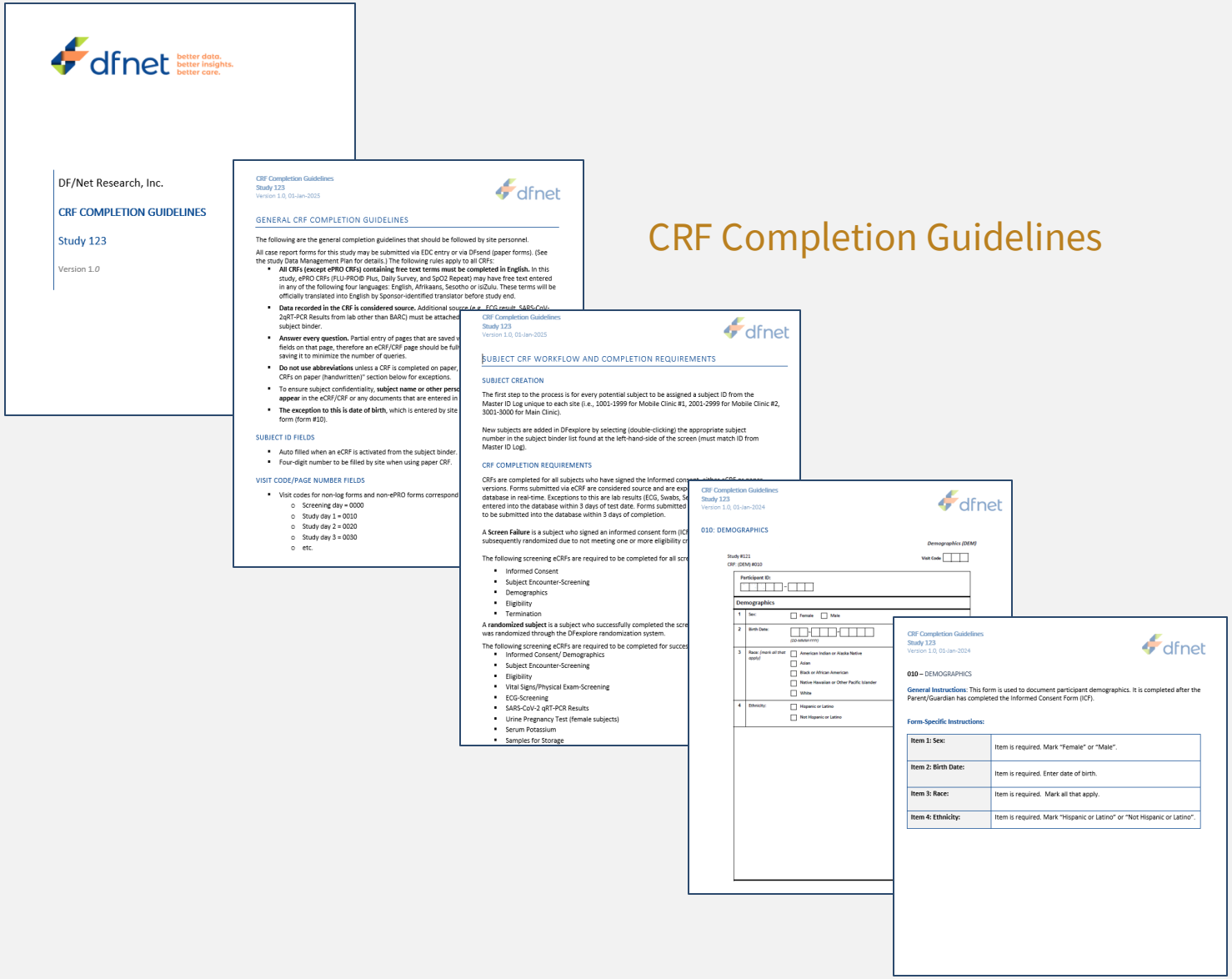
- Site, Monitor, Clinical Coder, etc.

Resources

- Virtual training sessions (recorded)
- Investigator/Study Team meetings
- Slides
- Videos (e.g. DFnet Academy)
- CCGs, DMP, etc.

6 Best Practice:

Ensuring Data Quality

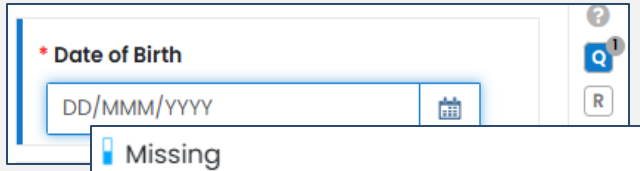


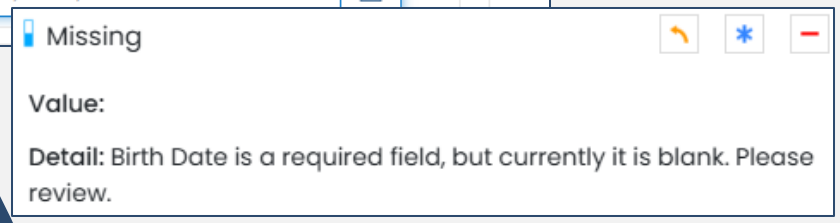
CRF Completion Guidelines

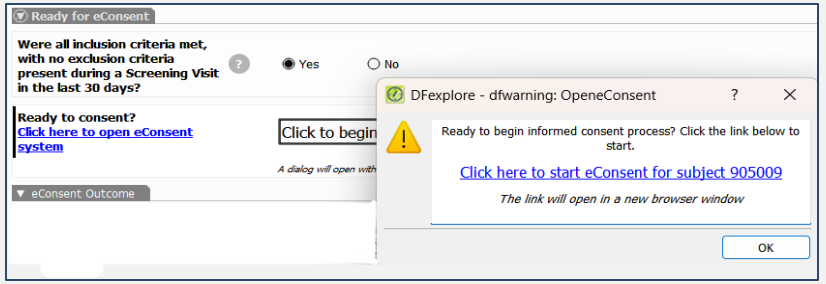
6 Best Practice:

Ensuring Data Quality

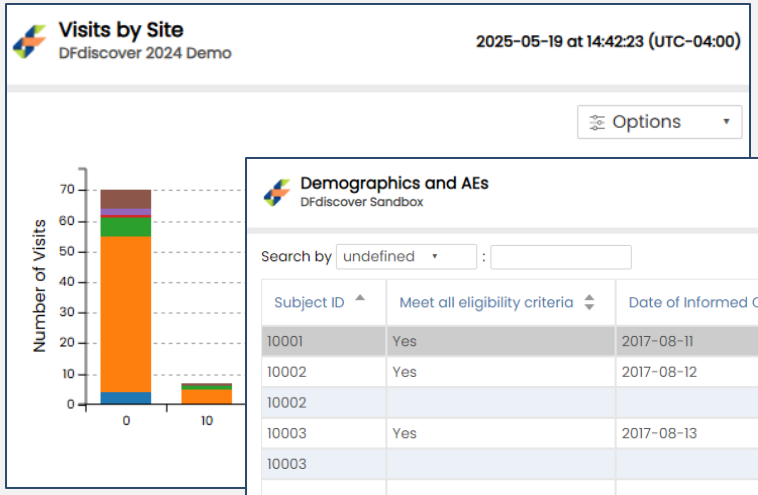
Built-in/Custom Edit Checks

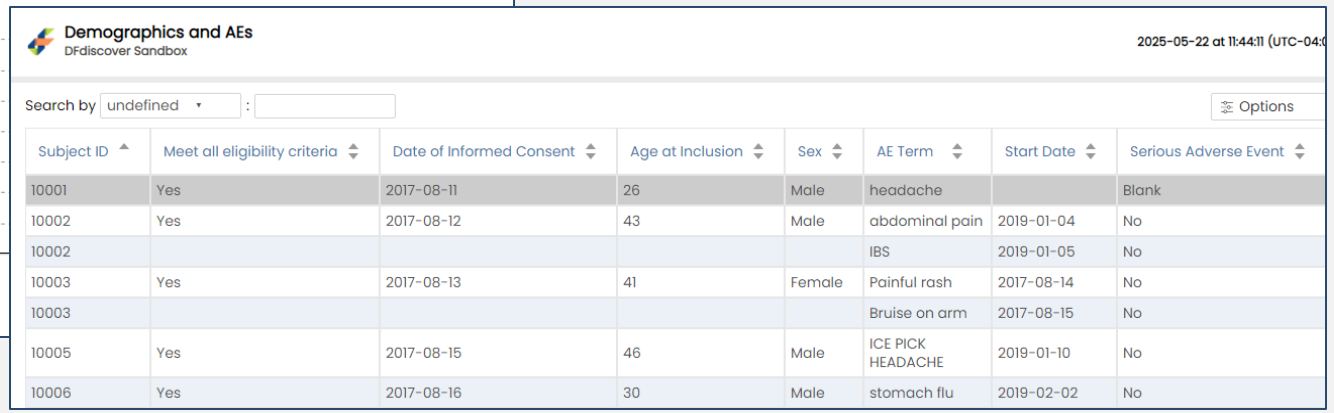






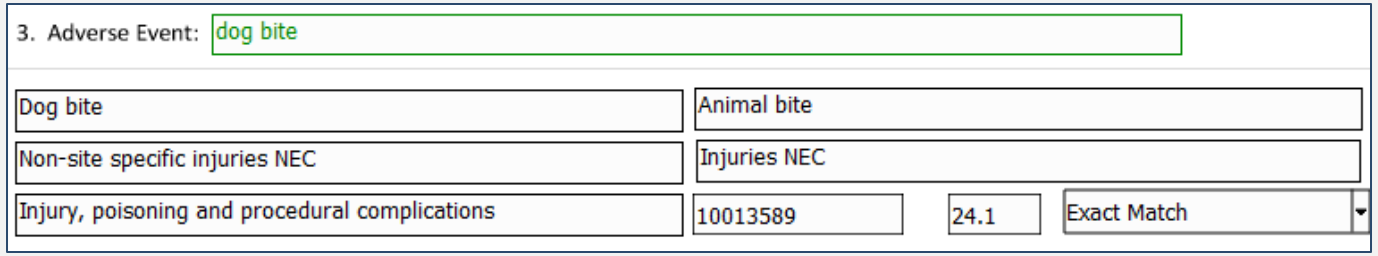
Standard/Custom Reports





Subject ID	Meet all eligibility criteria	Date of Informed Consent	Age at Inclusion	Sex	AE Term	Start Date	Serious Adverse Event
10001	Yes	2017-08-11	26	Male	headache		Blank
10002	Yes	2017-08-12	43	Male	abdominal pain	2019-01-04	No
10002					IBS	2019-01-05	No
10003	Yes	2017-08-13	41	Female	Painful rash	2017-08-14	No
10003					Bruise on arm	2017-08-15	No
10005	Yes	2017-08-15	46	Male	ICE PICK HEADACHE	2019-01-10	No
10006	Yes	2017-08-16	30	Male	stomach flu	2019-02-02	No

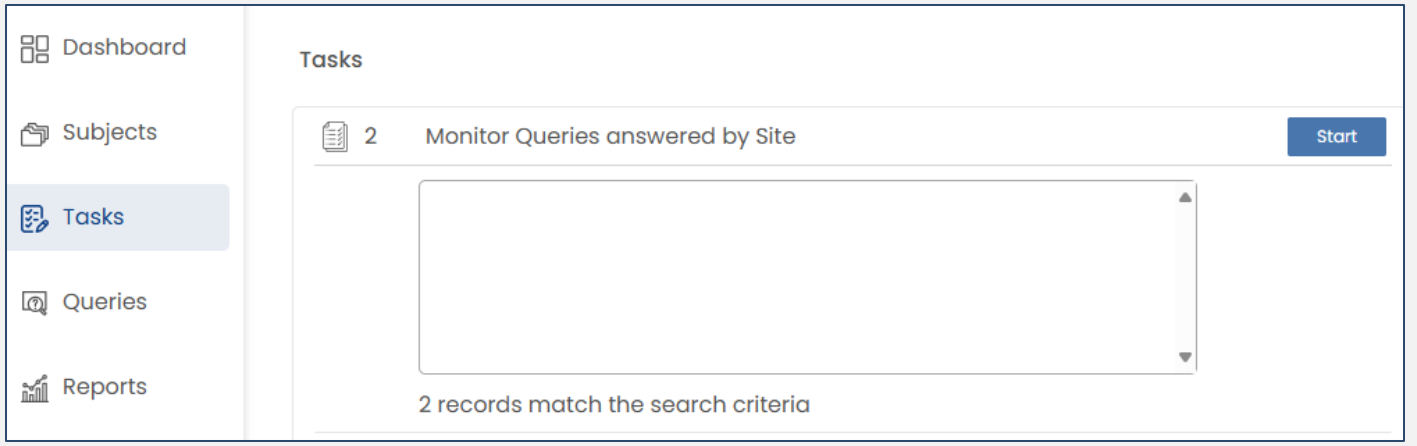
Clinical Coding



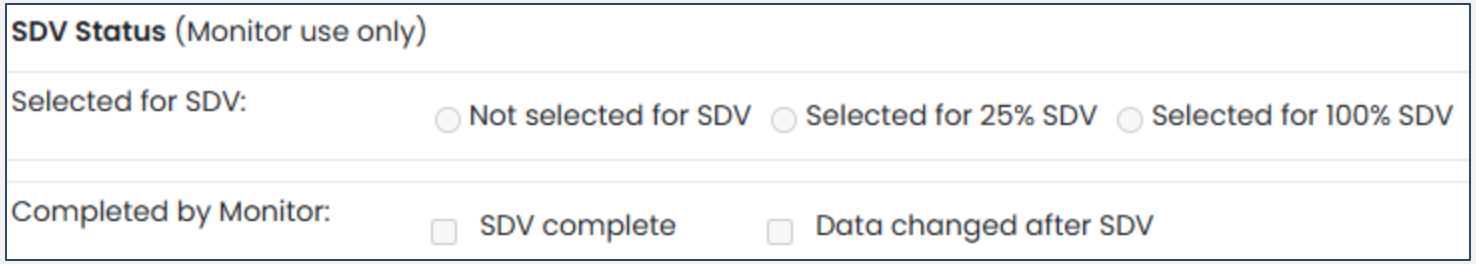
6 Best Practice:

Ensuring Data Quality

Tasks



Remote/On-site Monitoring



Data Reconciliation

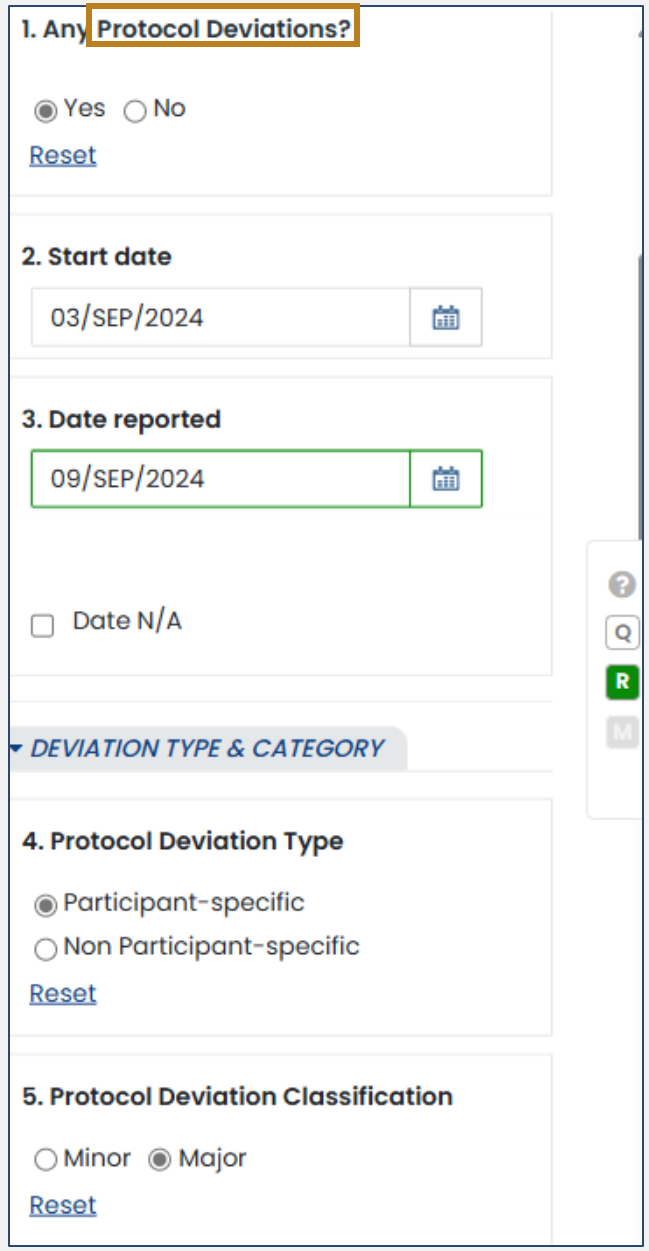
DFVISIT_LABEL	SUBJID	)RRES.DFVAR_DI	LBORRES	LBORRESU	.ES.DFVAF	LBORRES	LBORRESU
Supp #1 after Day 1 (01S)	P02ABP9906	Platelets	000123	g/dL	WBC	00045	mg/dL
Visit 02 Day 8	P02ABP9906	Platelets	000123	g/dL	WBC	00045	mg/dL
Supp #1 after Day 8 (02S)	P02ABP						
Supp #1 after Day 1 (01S)	P02ABP						
Visit 02 Day 8	P02ABP						
Supp #1 after Day 8 (02S)	P02ABP						
Supp #1 after Day 20 (03S)	P02ABP						
Supp #1 after Day 1 (01S)	P02ABP						
Visit 02 Day 8	P02ABP						
Supp #1 after Day 8 (02S)	P02ABP						
Supp #1 after Day 20 (03S)	P02ABP						

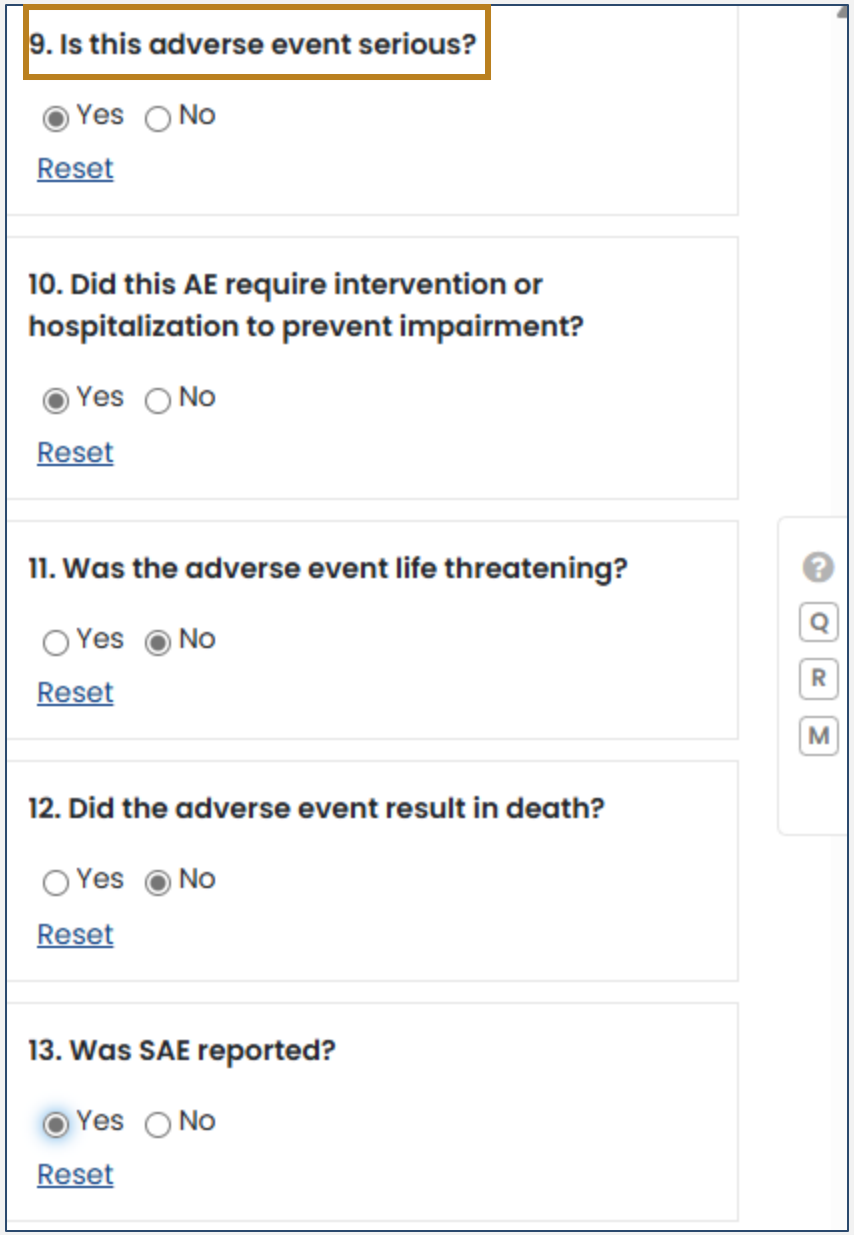
	A	B	C	D	E	F	G	H	I
1	Study	Visit	Subject	Test	Result	Unit	Test	Result	Unit
2	185	Supp #1 after Day 1 (01S)	P02ABP9906	Platelets	123	g/dL	WBC	45	mg/dL
3	185	Visit 02 Day 8	P02ABP9906	Platelets	123	g/dL	WBC	45	mg/dL
4	185	Supp #1 after Day 8 (02S)	P02ABP9906	Platelets	123	g/dL	WBC	45	mg/dL
5	185	Supp #1 after Day 1 (01S)	P02ABP9907	Platelets	2	mg/dL	WBC	3	U/L
6	185	Visit 02 Day 8	P02ABP9907	Platelets	2	mg/dL	WBC	3	U/L
7	185	Supp #1 after Day 8 (02S)	P02ABP9907	Platelets	2	mg/dL	WBC	3	U/L
8	185	Supp #1 after Day 20 (03S)	P02ABP9907	Platelets	2	mg/dL	WBC	3	U/L
9	185	Supp #1 after Day 1 (01S)	P02ABP9908	Platelets	2	mg/dL	WBC	3	U/L
10	185	Visit 02 Day 8	P02ABP9908	Platelets	1	g/dL	WBC	2	U/L
11	185	Supp #1 after Day 8 (02S)	P02ABP9908	Platelets	3	U/L	WBC	4	mm3
12	185	Supp #1 after Day 20 (03S)	P02ABP9908	Platelets	2	mg/dL	WBC	3	U/L

6 Best Practice:

Ensuring Data Quality

Detailed reporting of special events (SAEs, Protocol Deviations, etc.)



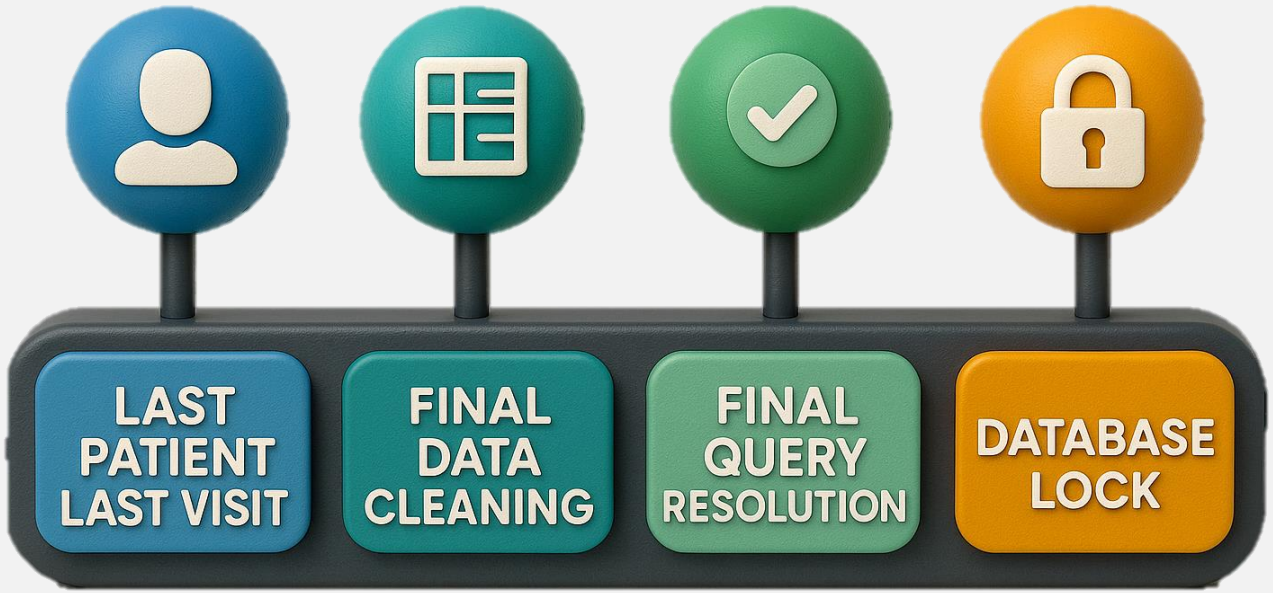


6 Best Practice:

Ensuring Data Quality

7 Best Practice:

Smooth Database Lock



Define a timeline and stick to it!

- Be sure everyone knows their part

Final data cleaning

- Integrity checks
- Critical variable review
- Data reconciliation

Implementing Best Practices

General Clinical Trial:

- ✓ Plan Early
- ✓ Standards and Compliance
- ✓ Choose Appropriate Data Capture Tools
- ✓ System Validation & Data Integrity
- ✓ Training
- ✓ Ensuring Data Quality
- ✓ Smooth Database Lock

Rare Disease Clinical Trial:

Solving Unique Challenges: Rare Disease Case Studies

Rare Diseases

What are Rare Diseases?

- **Rare**: Affecting < 200,000 individuals (US), or <1 in 2,000 in Canada, the European Union and majority of Africa.
- **Ultra-Rare**: Affecting <1 in 50,000
- Over 7,000 rare diseases exist → affecting over 300 million people globally.
- Globally affects more people than Cancer and AIDS combined
- Approximately 50% of people affected are children

Why Are Clinical Trials Crucial for Rare Diseases?

- ~95% of rare diseases lack approved treatments.
- Each trial is a building block
- Success is inspiring
- Push the boundaries of medical science
- Unite researchers, patients, advocates
- Provide hope to the 300 million+ affected

Key Challenges in RD Clinical Trials

Limited Patient Population

Complex & Heterogeneous Conditions
(variability in disease progression, confounders)

Geographical Spread

Regulatory Challenges
(specialized approval processes & compliance)

Underserved Locations with Limited Resources

Data Collection & Management Issues
(small sample sizes, data integrity concerns)

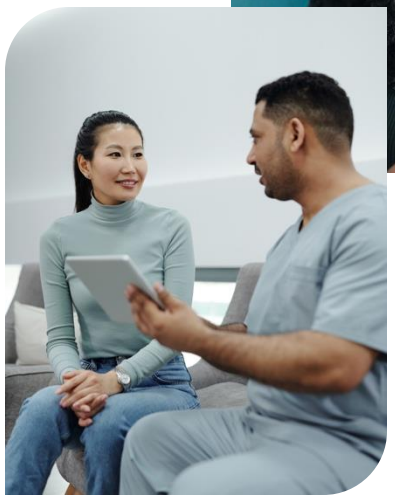
Overcoming Boundaries

DFnet Enabled:

- Decentralized trial in remote village
- No internet? No problem—offline data collection via DFcollect
- Rugged local intranet + satellite sync for secure uploads
- Source documents captured and redacted digitally
- Remote monitoring & 100% SDV without site visits

Cultivating Patient Connections

- Enhanced patient participation and engagement through innovative features
  - Creates communication pathway with subjects
  - Prioritizes patient safety
  - More convenient and less burden for participants
  - Improves data quality and protocol compliance
  - Makes the data immediately available (real-time)
  - Cost savings
- Built in multi-language feature facilitated global participation



Adaptive and Customizable

- Scalability and customization-tailored for small sample sizes and complex, adaptive protocols
- Mid-study changes implemented with no down-time, no disruption
- Agile team of experts

One rare disease trial added cohorts mid-study and underwent multiple protocol amendments. With DFnet's mirrored development database, we implemented updates instantly — no downtime, no data loss.

Ensuring Data Excellence

- Captured comprehensive data from multiple sources (EDC, ePRO, CRFs, and more)
- Maintained near lock-ready quality with real-time validation and tailored edit checks
- Delivered analysis-ready datasets (CDISC, SDTM, ADaM, MedDRA, WHO-DD compliant)

> *Our lock-ready data enabled interim analysis on 24-hour notice — critical for this adaptive, early-phase trial..*

Key Takeaways: Clinical Data Management in Rare Disease Trials

- **Rare disease trials demand precision** with small sample sizes, complex protocols, and evolving needs;
- **Clean, compliant data is critical** for timely decisions, patient safety, and regulatory success;
- **DFnet delivers end-to-end support** from protocol design through database lock;
- **Our platform adapts in real time** with no downtime, even during mid-study changes;
- You bring the mission. We bring the data strategy, tools, and team to get you there.

Questions?

Use the chat to ask a question or unmute to join the discussion.

Scan the QR code to take our short survey:

- Explore **past webinars and solutions** at dfnetresearch.com
- Watch for the **webinar recording** and a brief survey in your email
- We're here to support your next rare disease study.
- Contact us at info@dfnetresearch.com

MZANSI dfnet dfnet

South Africa | USA | Canada