Scientific Poster Session

n-lorem FOUNDATION

Nano-rare Patient Colloquium 2025

Monday, October 20 | 5:15 – 6:15 pm EST

Robust Safety Monitoring in n=1 Trials: A Scalable Approach from REDCap to PowerBI for Data Safety Monitoring Board Oversight

We present a scalable data lifecycle framework for n=1 trials, designed to support robust safety monitoring and Data Safety Monitoring Board (DSMB) review. Beginning with individualized protocol development, the workflow moves to custom REDCap electronic data capture (EDC) builds, remote data monitoring and cleaning, and direct export of cleaned data into PowerBI. Our PowerBI dashboards enable real-time, visualized safety review across adverse events, Concomitant medications, labs, and other key safety measures.

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Collecting Data from n=1 Trials

- Unique Challenges
  - Individualized protocols → heterogeneous datasets
  - Safety monitoring requires tailored data prioritization & visualization
- Our Framework
  - Scalable, end-to-end data lifecycle
  - Maintains flexibility for individualized patients, but also shared data templates for consistent data capture
  - Custom database for each patient using REDCap (Research Electronic Data Capture)
  - Remote monitoring & data cleaning
  - API-driven data flow into PowerBI dashboards
  - Quarterly Data Safety Monitoring Board (DSMB) review across multiple safety domains
- Goal
  - Enable accurate, timely, and intuitive safety review for decision-making

STEP 1 – Protocol Development

- Guided by n-Lorem Study Treatment and Assessment Review (STAR) Committee
  - Internal and external experts in clinical trial design, outcome measures, and assessments
- Each individualized protocol contains clearly defined clinical efficacy and safety assessments, assessment schedules, and data collection requirements

Study Visits (Days)	Baseline	Initial dose D1		Follow-up visit 1 D8	Second dose D28		Follow-up visit 2 D35	Third dose D84		Follow-up visit 3 D91	Fourth dose D168		Follow-up visit 4 D175	Maintenance Phase		
Study Window	45 Days	-		(+/- 3days)	(+/- 7 days)		(+/- 3days)	(+/- 7 days)		(+/- 3days)	(+/- 7 days)		(+/- 3days)	Doses every 84 days (+/- 7 days)		Follow-up 1-week post-dose (+/- 3days)
Location	Onsite	Onsite		Phone	Onsite		Phone	Onsite		Phone	Onsite		Phone	Onsite		Phone
		Pre-Dose[a]	Post-Dose[j]		Pre-Dose[a]	Post-Dose[j]		Pre-Dose[a]	Post-Dose[j]		Pre-Dose[a]	Post-Dose[j]		Pre-Dose[a]	Post-Dose[j]	
Informed consent	X															
Review eligibility	X															
Medical history[b]	X															
Ancillary Procedures	X															
Physical examination	X	X	X		X	X		X	X		X	X		X	X	
Neurological exam	X	X	X		X	X		X	X		X	X		X	X	
Clinical lab testing[c]	X	X			X			X			X			X		
Syde w ankle accelerometer[d]	X															
Gait analysis	X															
WCS	X										X			X[e]		
Vineland-3	X													X[f]		
BSID-4	X													X[f]		
ORCA	X										X			X[e]		
QI-Disability	X										X			X[e]		
ABC-C	X										X			X[e]		
RBS-R	X										X			X[e]		
Brain MRI[g]	X															
12-lead ECG	X	X			X			X			X			X		
Vital signs	X	X	X		X	X		X	X		X	X		X	X	
CSF Sampling[h]		X			X			X			X			X		
Study drug injection		X			X			X			X			X		
Adverse events		X														
Concomitant medications	X															

STEP 2 – Building a data collection database

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Customized

- Electronic case report forms (eCRFs) built based on protocol
- Visits and assessments mapped to study schedule
- Auto-calculations and data validation rules reduce entry errors

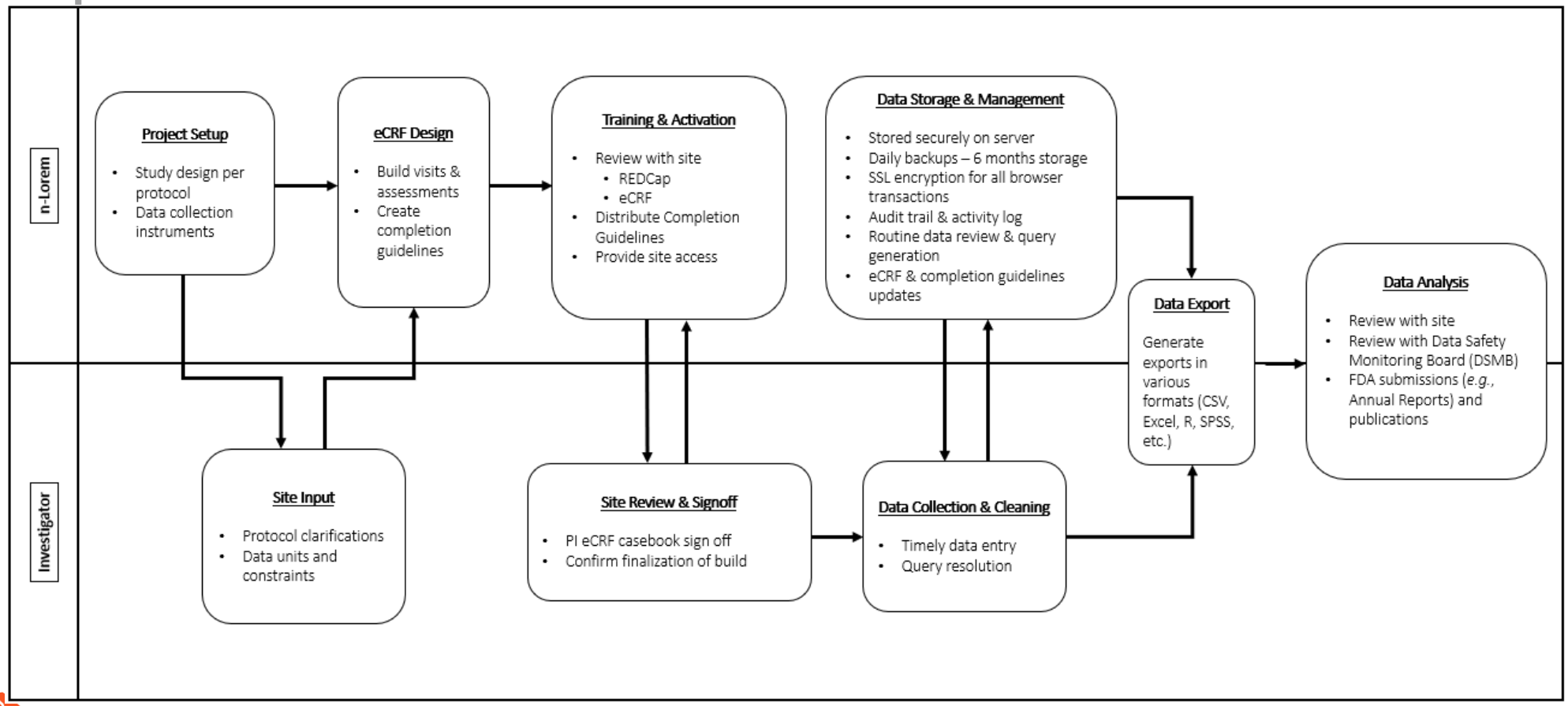
Collaborative

- Investigators and study teams provide input on site-specific assessments and data units
- Training of site members on database and data entry. PI sign off on database before it goes live.
- 24-hour reporting from site for serious adverse events (SAEs)

Consistent

- Standardized safety eCRFs
- Standardized efficacy eCRFs across patients with similar phenotype and protocol endpoints
- Completion guidelines ensure uniform data entry across sites

STEP 2 – Database workflow



STEP 3 – Reviewing and Cleaning the Data

- Adverse Events (AEs) – any new health issues a patient experiences during the trial
  - Database sends automatic alerts whenever new AEs are entered by site
  - Reviewed by n-Lorem team, with follow up discussion with the study doctor if needed
- Routine Reviews
  - Data reviewed every quarter to prepare for safety board meetings (DSMB)
- Queries – mistakes or missing data
  - Queries manually generated by n-Lorem and issued to sites for resolution
  - Database API integration for automated daily query tracking dashboard

STEP 3 – Reviewing and Cleaning the Data

Sample database for review

Data Collection Instrument	Baseline	Dose 1	Dose 1 Follow Up	Dose 2	Dose 2 Follow Up	Dose 3	Dose 3 Follow Up	Dose 4	Dose 4 Follow Up	Dose 5	Dose 5 Follow Up	Dose 6	Dose 6 Follow Up	Dose 7	Dose 7 Follow Up	Dose 8	Dose 8 Follow Up
Review Eligibility	●																
Demographics	●																
Medical History	● +																
Initial Genetic Testing	●																
Ancilliary Procedures																	
Seizure Diary	●	●		●		●		●		●		●		●		●	
Physical Examination	● +	● +		● +		● +		● +		● +		● +		● +		● +	
Neurological Examination	● +	● +		● +		● +		● +		● +		● +		● +		● +	
Clinical Safety Laboratory Collection	●	●		●		●		●		●		●		●		●	
Brain MRI	●											●					
BSID4	●											●					
ABC	●	●		●		●		●		●		●		●		●	
Vineland 3	●	●		●		●		●		●		●		●		●	
Vineland 3 - Teacher	●	●		●		●		●		●		●		●		●	
ORCA	●	●		●		●		●		●		●		●		●	
Dyskinetic CPFIS	●	●		●		●		●		●		●		●		●	
Quality Of Life	●	●		●		●		●		●		●		●		●	
Bristol Stool Scale	●	●		●		●		●		●		●		●		●	
Home Gait Analysis												●					
ECG	●	●		●		●		●		●		●		●		●	
Vital Signs	● +	● +		● +		● +		● +		● +		● +		● +		● +	
EEG	●	●		●		●		●		●		●		●		●	
Study Drug Administration		●		●		●		●		●		●		●		●	
Adverse Events																	
Concomitant Medications																	
Follow-up			●		●		●		●		●		●		●		●

STEP 4 – Exporting data to dashboard

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Feeds

- Cleaned databases connect via API to a PowerBI dashboard for each patient
- Each patient has their own dashboard with tabs
- Dashboards display individualized safety biomarkers and visualizations

Format

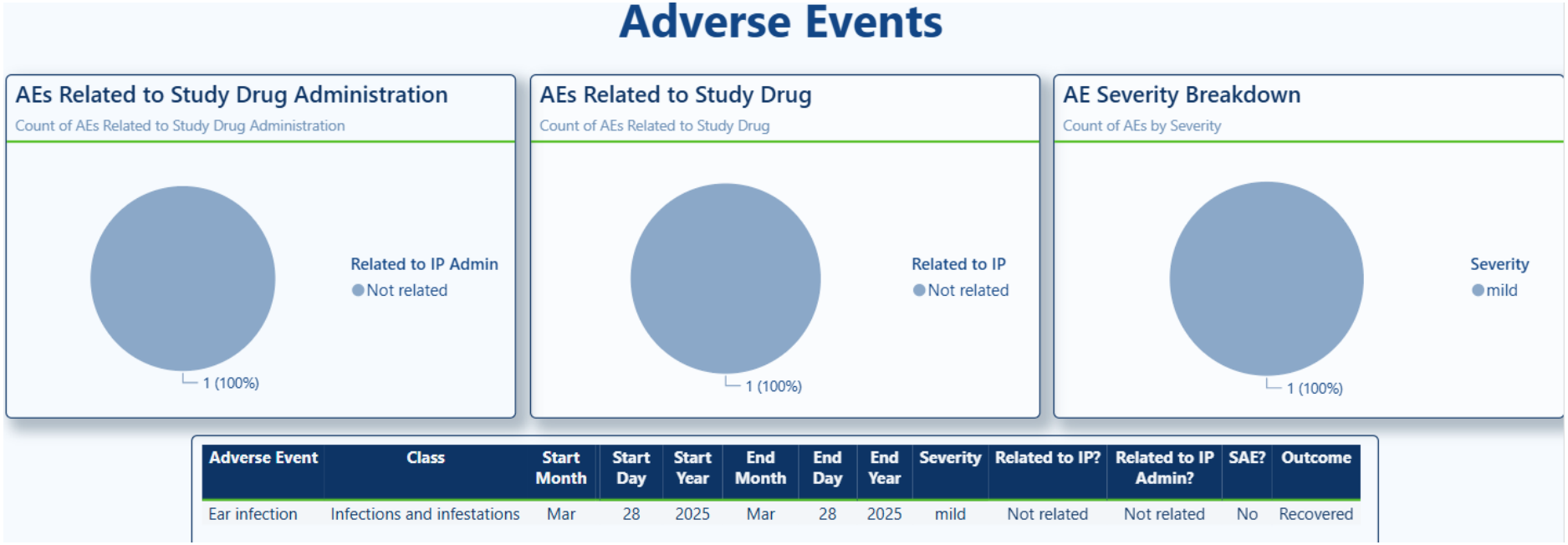
- Dashboard patient cards with total number of doses, highest dose level, number of AEs, and latest vital signs (blood pressure and heart rate)
- Data models structured so safety domains—AEs, concomitant medications, labs, vitals—can be easily reviewed by category

Flags

- Automated highlighting of abnormal values
- Trend detection using data visualization
  - Vital signs line graphs for systolic and diastolic blood pressure
  - AE pie charts to see distribution of mild vs moderate vs severe AEs

STEP 4 – PowerBI Dashboard

Sample AE tab in a patient's dashboard for safety review



Adverse Event	Class	Start Month	Start Day	Start Year	End Month	End Day	End Year	Severity	Related to IP?	Related to IP Admin?	SAE?	Outcome
Ear infection	Infections and infestations	Mar	28	2025	Mar	28	2025	mild	Not related	Not related	No	Recovered

STEP 5 – Data Safety Monitoring Board

- Independent subject matter experts for unbiased oversight
  - External and internal specialists in ASO technology, clinical trials, drug development, and nano-rare diseases

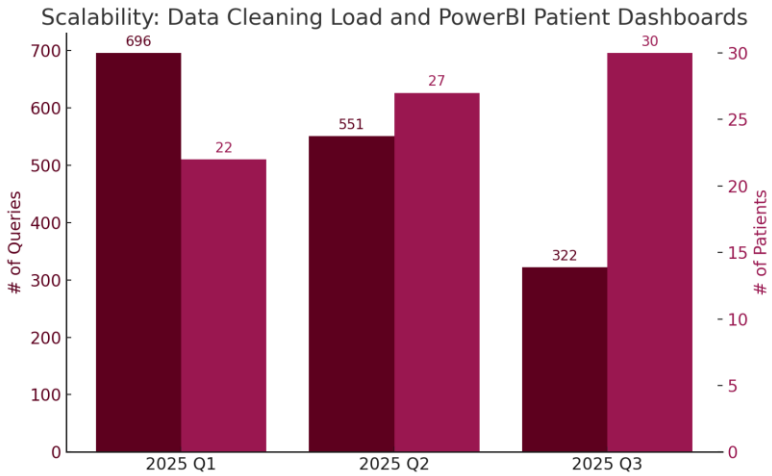
- Quarterly Reviews
  - Cleaned safety data in PowerBI dashboards
  - PowerPoint summaries (aggregate safety data, updates since last meeting)
  - Support timely, informed safety recommendations (*e.g.* recommendation to stop dose escalation until AE resolves)

Conclusions

- Implemented for 30 patients as of Aug 1, 2025, with >10 more expected by year-end

- Fine-tuned process
  - Efficient collection and management of high-quality trial data

- Standardized yet customizable workflows:
  - Support timely safety oversight
  - Enable clear, data-driven decisions
  - Allow efficient replication across future n=1 trials



We extend our sincere gratitude to the physicians and study teams at the treating institutions for their dedication and collaboration. We are especially grateful to the patients and families for their trust, commitment, and participation.

Thank you

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