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Digital Endpoint Data Integrity Challenges in Clinical Trials: Build vs Buy Decision

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Digital Endpoints in Clinical Trials

Digital endpoints, readouts of drug efficacy derived via **digital health technologies (DHTs)**, are increasingly being adopted in clinical trials for allowing real-time monitoring, deeper and broader data collection, and decentralization of clinical trials.

- **Wearable devices:** heart rate, sleep pattern
- **Remote monitoring tools:** blood glucose level, blood pressure
- **Patient-reported outcomes/tests:** tablet-based cognitive drawing tests

Data Integrity Challenges

To impact drug development, endpoints require extensive validation to insure they faithfully capture a meaningful aspect of patient health. Currently, digital endpoints can suffer from **low-quality and/or incomplete data** due to incorrect usage of DHTs, lack of quality metrics, and inefficient communication. There is an opportunity to mitigate this issue and speed up validation efforts.

Case Study: Novartis ALS Study using DHTs

- Amyotrophic lateral sclerosis (ALS) leads to progressive muscle weakening and mortality within 3-4 years. The ALS functional rating scale-revised (ALSFRS-R) is widely used as an endpoint but is limited in sensitivity and linearity.
- A Novartis study examined home-based digital tools aligned with ALSFRS-R functional domains, using a unified vendor application for data integration. The study team created an RShiny application for data monitoring.
- **Challenges**: Technical problems, inconsistent patient participation, complex issue detection requiring advanced analytics and expert involvement for data validation, communication barriers due to indirect patient interaction, and scalability concerns with RShiny tool.
- **Opportunity identified**: Potential for up to 70-80% **automatic resolution** of quality issues in hindsight, if experience from current study applied to future use of these DHTs.

Project Objectives and Methodology

Goal: analyze and make recommendations based on two competing strategies for efficient DHT data monitoring: "buy" vs. "build".

"Build": Develop internal software tools and processes for automated data quality checks and communication

"Buy": Mandate vendor compliance with quality metrics and issue notifications

The final recommended framework should be **scalable**, **easy to implement**, and **minimize burden** to study teams, clinical sites, and study participants.

To maximize the richness and depth of information gathered, our main research methodology includes **secondary research**, **unstructured interview**, and **structured questionnaire**.

Results 1: Internal Interview Findings

Complex information flow involving multiple stakeholders (clinical trial team members, clinical trial site staff, and digital endpoint experts) is required, in addition to having established quality metrics.

A comprehensive internal solution is already deployed in parts of the organization, but **requires human monitoring and intervention**. More automated solutions are welcome but the organization is sensitive to **cost**, as well as **changes to existing infrastructure**.

Team identified key requirements for a comprehensive solution:

- **Objective detection of missing/out of range values**
- **Automated notification of clinical trial site staff**
- **Configuration to know when data are expected**
- **Minimal expert oversight**

Results 2: Data Management Vendor Interview Findings

Unstructured interviews with five data management contract research organizations (CROs) revealed that each **now offered a solution matching the identified requirements**. However, added costs could be substantial, and real-world performance has not yet been explored.

Results 3: DHT Vendor Questionnaire Findings

The team also reached out to vendors already providing DHT services in Novartis early phase trials. Some vendors offer solutions that meet identified requirements, while others are actively pursuing capabilities.

DHT Platform Capabilities	# of vendors
DHT data ingested <24h from generation	6/6
Data access via application program interface (API)	5/6
Objectively detects missingness or out-of-range values	5/6 1 under development
Automatically notify site and patient when data quality issue arises	4/6
Time to notify site/patient when data quality issue arises	Real-time to 7 days
Know when data are expected	4/6 1 under development

Final buy and build Recommendation and Next Steps

	Recommendation	Potential Next Steps
Buy	• Deploy new capabilities of CRO/DHT vendors	• Validate "buy" hypothesis with pilot
Build	• Enhance DHT vendor vetting for trials • Increase communication across Novartis teams with similar roles across different phases of drug development	• Share present results with internal qualification teams • Share internal capabilities, e.g.apps & dashboards