PicnicHealth

RWD147

Quantifying the Effectiveness of a RWD Quality Framework; A Case Study Using Paroxysmal Nocturnal Hemoglobinuria (PNH)

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Background + Objective

Background

- Clinical trials have historically been regarded as the gold standard of high-quality data in research
- Real-world data (RWD) offers valuable nuance and efficiency and has been increasingly incorporated into clinical studies
- Many frameworks have been developed for conducting research using RWD to define criteria for assessing data quality and ensuring rigorous research practices

Objective

- We applied a quality framework to evaluate the reliability of data in a dataset for Paroxysmal Nocturnal Hemoglobinuria (PNH), a rare blood disorder, by conducting plausibility, conformance, and clinical relevance checks

Methods

Data Source

- Patient recruitment began in January 2020, with the latest enrollment in February 2023
- Patients with PNH provided consent to participate in the PicnicHealth Research Platform for the collection of their medical records from U.S. health systems
- Data was abstracted in 3-month intervals from structured and unstructured medical records using human-in-the-loop AI

Methodology

- We adapted an established quality framework (Duke Margolis Center for Health Policy) to assess data reliability, which consisted of checks for:

Plausibility:	An assessment of the clinical and logical believability or truthfulness of data values
Conformance:	Data structure is as expected and data are within predefined internal, relational, formatting, and computational standards; variables that should be the same across export tables are in fact the same
Clinical Relevance:	Dataset has expected demographic distributions, population has expected clinical characteristics

- We applied programmatic rules designed to identify potential plausibility, conformance, and clinical relevance quality issues across data domains (ex: conditions, observations, labs, etc.)
- Descriptive statistics were reported for key data elements

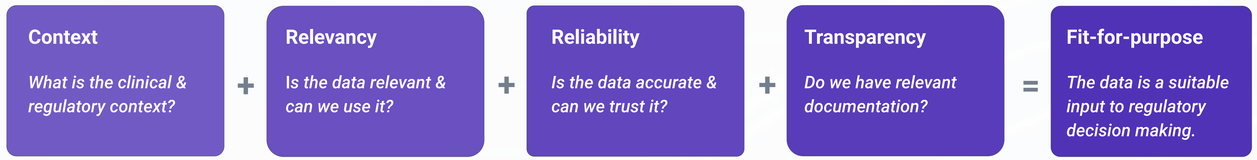

Figure 1: Schematic of PicnicHealth's quality framework. This poster focuses on the "reliability" component of the framework.

Table 1. Demographics and clinical characteristics of patients with PNH included in this study.

	Overall (N=90)
Age at diagnosis	
Mean (SD)	30.4 (11.3)
Median [Min, Max]	28.3 [13.6, 67.5]
Sex	
Female	63 (70.0%)
Male	27 (30.0%)
Race	
Asian	5 (5.6%)
Black or African American	11 (12.2%)
More than one race	5 (5.6%)
White	62 (68.9%)
Unknown	7 (7.7%)
Ethnicity	
Hispanic or Latino	11 (12.2%)
Not Hispanic or Latino	76 (84.4%)
Unknown	3 (3.3%)
Years between diagnosis and enrollment	
Mean (SD)	7.94 (8.04)
Median [Min, Max]	5.84 [0, 33.1]
True diagnosis date known	
Yes	80 (88.9%)
No*	10 (11.1%)
Precision of diagnosis date	
Day	32 (35.6%)
Month	35 (38.9%)
Year*	23 (25.6%)

**Reviewed for additional records to request*

Table 2. Distribution of quality rules applied to the PNH cohort by rule type.

Rule type	Example rule	Domain of example rule
Disease-agnostic plausibility	Drug eras for a patient should start and end after their diagnosis date	Drugs
Disease-agnostic conformance	Patients should not have data before their birth date	Person
PNH-specific plausibility	PNH Breakthrough hemolysis should occur inside treatment period	Condition, drug
Clinical relevance	Identifying high LDH values for clinical review of related outcomes/procedures. ex: Breakthrough hemolysis events should have LDH or hemoglobin value +/- 3 days around event date and during any treatment (Figure 2). Hemolysis events without recent LDH values are identified for clinical review	Condition, procedures, measurements

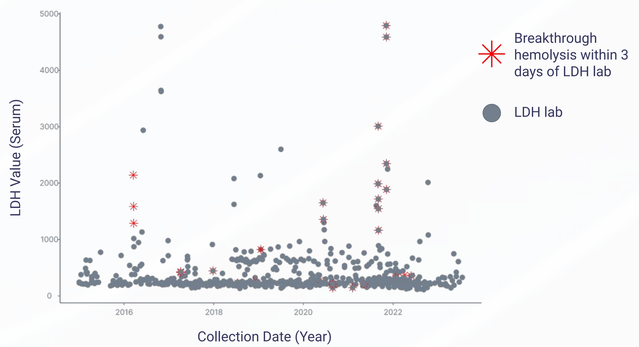

Figure 2: Scatterplot of serum/plasma LDH lab values over time, with lab values collected within 3 days of a breakthrough hemolysis event in red.

Results

- The PNH cohort included 90 patients
  - 70% female
  - 69% White, 12% Black, and 12% Hispanic or Latino
- 222 median number of records per patient, with 10 (6, 14) median (IQR) years of available records
- We deployed 143 disease-agnostic plausibility and conformance quality rules and 19 rules specific to the PNH dataset
  - After abstraction and processing 3 months of new records, 105 errors were identified
    - 96 disease-agnostic errors
    - 9 PNH-specific errors
  - Each error was reviewed by a clinician for data correction or justification of why the data should not be corrected
  - Most common errors included conditions before diagnosis (breakthrough hemolysis, hemoglobinuria, renal vein thrombosis, thrombosis) and outliers of vital signs
    - Typical causes of errors included errors due to missing records (submitted request for additional records), abstraction errors (corrected by clinician), and errors in the source medical record (dismissed with note from reviewer)

Conclusions

- Errors highlighted potential anomalies in a patient's care journey, allowing clinicians to determine whether there was an abstraction error, a error in the source medical record, or there were additional records that could be requested to provide a more complete picture of a patient's care
- Data corrections were tracked and traceable to source records, resulting in improved data provenance and higher data quality
- These findings informed abstractor retraining efforts for future work and improved human-in-the-loop AI model performance
- Implementing a quality framework for RWD, which incorporates logical checks at various stages during the dataset generation process, leads to the creation of a higher quality dataset

Acknowledgements & Disclosures

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- The authors are employees of PicnicHealth