Leveraging Real-World Evidence for Rare Disease and Oncology HTAs:

Insights from Pulse Infoframe's Hybrid Approach

Authors

Femida Gwadry-Sridhar1, Daniel Lewi1, Kathleen Coolidge1,

Affiliations

1. Pulse Infoframe, Inc., London, ON, Canada;

Contact Information

contact@pulseinfoframe.com

Introduction

Health technology assessments (HTAs) in rare disease and oncology face challenges in demonstrating payer value due to small patient numbers and limited data availability. Often endpoints that consider patient reported outcomes and preferences are also not consistently collected.

To address these issues, Pulse Infoframe collects data from sites and directly from patients using a hybrid approach. This methodology integrates multi-real-world data sources (RWD) that are curated and harmonized to support HTA decisions. This pragmatic data strategy, enables more efficient data collection to consider patient relevant outcomes that are key to technology assessment decisions.

Challenges

Real-World Data

Fills data gaps with patient-reported outcomes.

Data Scarcity

Limits reimbursement and regulatory approval.

Patient-First Approach

ensures HTA decision-making aligns with real-world needs

Clinical Trials

Fail to reflect real-world outcomes and patient experiences

Analysis

Overcoming HTA Barriers with Real-World Data

A hybrid data collection model combining site-based and decentralized methods ensures robust evidence generation.

Patient preferences and outcomes are integrated to refine endpoints and improve the relevance of HTA evaluations.

Conclusion

The path to accessing life-saving therapies can be complex. Pulse Infoframe's approach to RWE in rare diseases and oncology demonstrates how integrating patient-centric data with HTA requirements can bridge the gap between clinical trials and real-world application.

By aligning patient needs with regulatory priorities, RWE enhances informed decision-making, ultimately improving access to vital therapies for patients worldwide.

Case Studies

Uveal Melanoma

Ocular Melanoma Natural History Study

Objective: Demonstrate unmet medical need to support pricing and reimbursement. Collect data to support health technology assess post pivotal trial.

Registry Overview:

One of the most comprehensive global ocular melanoma registries, capturing real-world diagnostic, treatment, and outcome data.

Designed to reflect the usual car paradigm and patient outcomes in the absence of therapeutic choices.

The data also characterized a better understanding on research on emerging therapies, prognostic biomarkers, and long-term progression fere and overall survival .

Pulse Infoframe's Role:

Leading site based retrospective and prospective data collection, prior to treatment availability and then post availability through early access programs. including from early access programs and additional data sources.

Ensured high-quality data integration, standardization, and accessibility for research.

Facilitated targeted subpopulation analyses to support comparator arms and observational studies.

Outcomes:

- Longitudinal data highlighted through natural history disease progression in untreated patients, reinforcing the unmet need.
- Enabled comparative effectiveness research across different treatment strategies.
- Supported successful reimbursement approvals in multiple regions.
- Registry site network has expanded to 6 countries across 3 continents

Lung Cancer

Phase IV pricing & reimbursement support

Objective: Support pricing dossier and regulatory approval for a sponsors approved NSCLC drug.

Registry Overview:

North American real-world evidence initiative studying lung cancer patients using IASLC data requirements

Collects data on patient outcomes, treatment patterns, and disease progression.
A specific study conducted to illustrate unmet need in EGFR and ALK+ patients outlined in the POTENT study

Provides critical insights for oncologists, researchers, and health technology assessment (HTA) bodies.

Pulse Infoframe's Role:

Managed implementation, ensuring compliance across regulatory environments.

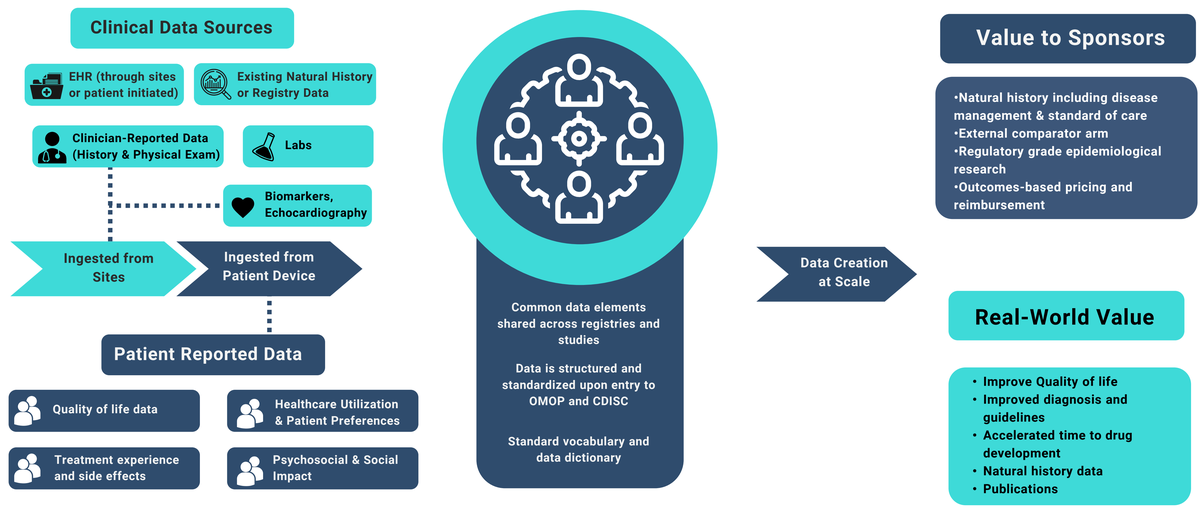
Maintained tight timelines and streamlined operations across multiple sites with tailored training.

Employed proactive monitoring to optimize data quality and minimize delays.

Outcomes:

- Supported pricing dossier and regulatory approval, enabling successful market access.
- Identified a considerable unmet need in a large patient population.
- Provided robust real-world data for sub-analyses, including diverse and underrepresented groups.
- Published two manuscripts in top tier journal and a poster at ESMO

Pulse Infoframe's Hybrid Data Collection Approach



Adjuvant Crizotinib in Uveal Melanoma

frontiers in Oncology

PD-L1 & Outcomes in Early NSCLC

Current Oncology

Real-World EGFR+ NSCLC Outcomes

ESMO

Learn more about Pulse Infoframe's Oncology registries