Archival Sample Testing for Real-World Insights: Innovative Approaches to Enriching Retrospective Studies with Biomarker Data

RWD18

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OBJECTIVES

- Precision oncology targets treatment to specific subpopulations characterized by their molecular profile, frequently evidenced by distinct biomarker expression.
- Guidelines recommend testing for biomarkers that inform treatment choices. Implementation of testing guidelines in clinical practice is often slow to scale across complete healthcare economies.
- Additionally, emerging biomarkers often crucial for the widespread adoption of new treatments, are usually under-tested in clinical practice and consequently under-reported in real-world data, creating evidence gaps.
- This research describes how testing of archival biosamples across 10 indications, using IQVIA's Oncology Evidence Network (OEN), provides robust biomarker insights not usually available, providing crucial evidence for Health Technology Assessments and regulatory submissions.

METHODS

- Extensive site selection processes identified suitable sites, using criteria of: comprehensive biobanks, research-aligned pathology services, validated testing facilities and integrated electronic medical record (EMR) data (Figure 1).
- Harmonized global protocols defined the detailed clinical EMR patient data to be linked with archival biosamples and likely screening cohort sizes required to achieve the necessary biomarker positivity.
- All sample testing procedures adhered to standardized quality control measures as defined in a laboratory manual applicable across all sites and laboratories.
- Advanced analytics and cross-site harmonization ensured robust data integrity and reproducibility throughout the study.

RESULTS

- Tumor biopsies or blood samples were available for testing at 23 sites/networks across 6 European countries and the United States of America (Figure 2).
- 2404 samples were processed for testing: 1956 samples were tested locally (19 sites) and the remaining samples were shipped as microdissection slides or blocks for testing in a central laboratory (4 sites). Valid results were obtained for 2031 samples (84%).
- The attrition rate for unusable samples across all sites was 16% (range 0% to 48%). The main reasons for attrition were an insufficient residual volume of tissue for clinical use (reducing willingness to use sample for research) and inadequate sample quality for definitive biomarker testing.
- The total study duration was 2 years, using archival tissue retrieved from a 6-year period and already available patient follow-up data (0.5 years for sample testing and 1.5 years for setup and analysis).
- It was estimated that the same scale of cohort gathered using a traditional prospective design would have required a 6-year study (3 years for sample collection, 0.5 years for follow-up, and 1.5 years for setup and analysis).

Known Precision Oncology challenges	
	How do we enable **sufficiently powered studies** in 'rare' populations?
	Who is **testing** for what, how and where?
	How do we characterise **biomarker-defined populations** where testing isn't performed?
	How can we efficiently collect data across tumour types for **tumour-agnostic indications**?

Figure 1 Oncology Evidence Network (OEN) facilitates comprehensive access to tissue testing capabilities with linked clinical data

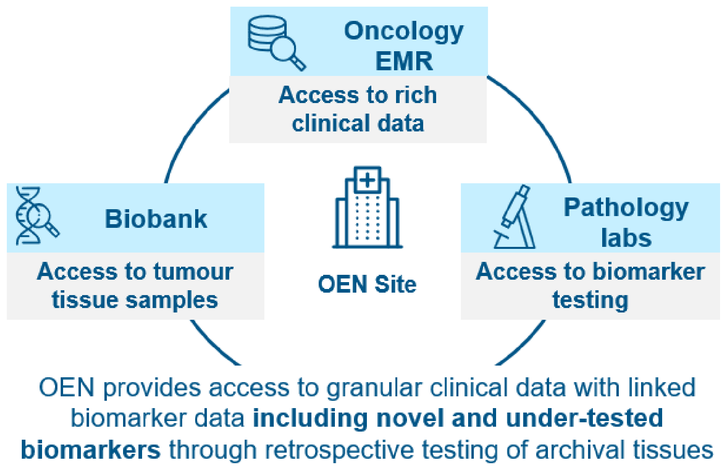
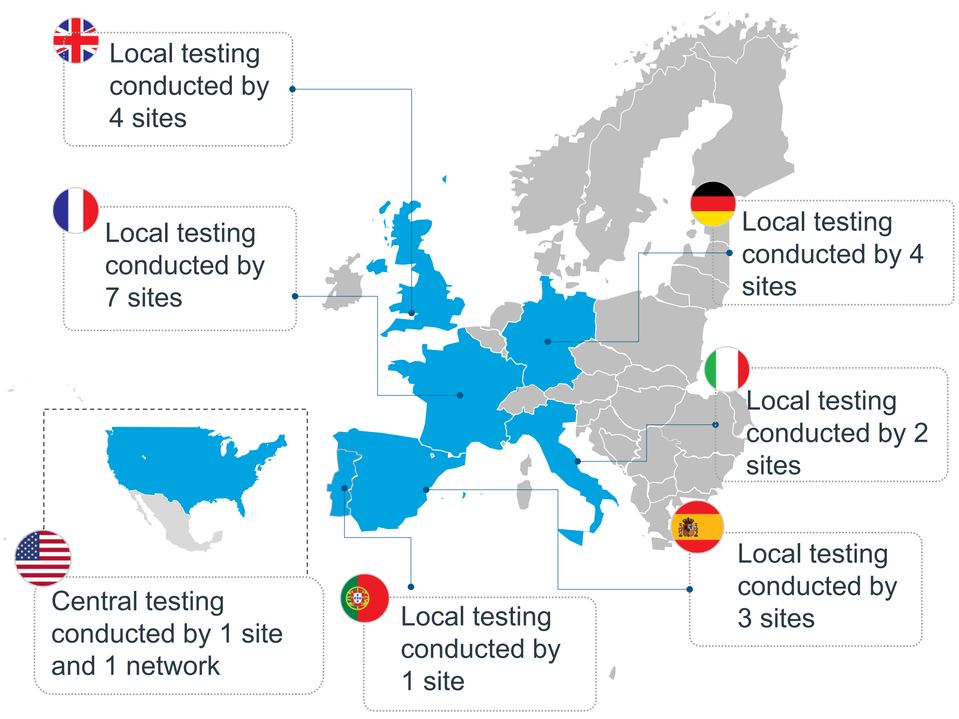
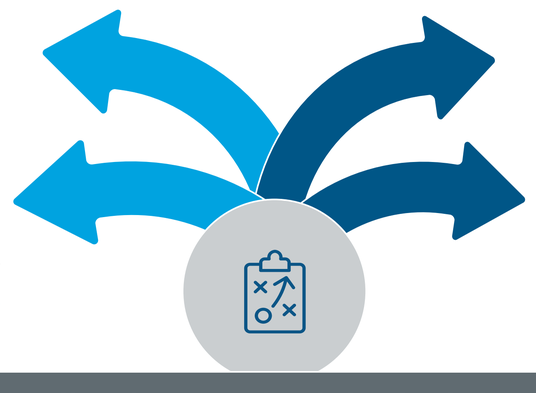


Figure 2: Geographical distribution of testing sites



Essential insights



Strategic Selection of Testing Modality

The choice between centralized and local testing is pivotal. Centralized testing offers standardization and analytical consistency, while local testing can accelerate timelines and leverage site-specific expertise. Optimal strategy selection requires nuanced consideration of cost structures, logistical complexity, regulatory environments, and the technical capabilities.

Cohort Size and Patient Attrition

Initial feasibility can overestimate the number of eligible samples. Attrition arises from factors e.g. sample age, quality degradation, and incomplete samples. Stringent inclusion/exclusion criteria and evolving data privacy regulations can substantially reduce the final cohort. Robust pre-study audits and dynamic feasibility models are essential to mitigate these risks.

Ethical and Regulatory Frameworks

Tissue testing RW-settings demands rigorous adherence to ethical standards, which vary across jurisdictions. Key considerations include informed consent, patient information disclosure, and clarifying study classification (interventional vs. non-interventional) with local ethics committees. Proactive engagement with regulatory bodies and transparent communication with sites are critical to ensure compliance and maintain trust.

Data Integration and Quality Assurance

Linking biomarker results with comprehensive clinical data requires sophisticated data management infrastructure and harmonization protocols. Ensuring data integrity across sources is fundamental for generating actionable RWE that meets the expectations of HTA bodies and regulatory agencies.

These learnings underscore the necessity of expert coordination, methodological rigor, and adaptive strategies to unlock the full potential of archival tissue testing for precision oncology research.

CONCLUSIONS

- **Leveraging archival tissue testing across an international network enables the efficient generation of robust, clinically relevant real-world evidence for precision oncology – a core use case is the population of retrospective control cohorts defined by emerging biomarkers that are not yet tested in routine care.**
- **Our innovative approach accelerates actionable insights for new therapy launches, supporting timely and informed decision-making in diverse tumor types and geographies as well as addressing critical evidence gaps for health technology assessments and regulatory submissions.**
- **The IQVIA Oncology Evidence Network provides a robust foundation for leveraging established partnerships with clinical sites and biobanks, enabling ethically compliant access to high-quality biosamples and clinically rich data – this has resulted in large-scale, collaborative real-world research initiatives that are significantly more efficient than a prospective approach.**

ISPOR Europe 2025, Glasgow