White Paper

Leveraging Real World Evidence to Support Oncology Targeted Therapies

Table of contents

Executive summary

IQVIA's Oncology Evidence Network (OEN) collaborates with academic hospitals and cancer centers to produce significant real-world evidence studies in oncology. With more than 30 partners, OEN has enabled many RWE studies across a broad range of oncology indications, including natural history, comparative effectiveness, external comparator, and precision oncology studies.

Our white paper provides an overview of a comprehensive solution for generating Real World Evidence (RWE) in the field of precision oncology.

Oncology targeted therapies can significantly benefit from RWE programs, **speeding up clinical development, supporting market access, and driving market penetration.**

However, the **adoption of novel biomarkers in clinical practice is slow.** This, combined with the **variability in testing practices** and the **low prevalence of some novel biomarkers,** makes RWE programs unpredictable, costly and even unfeasible in certain cases.

Our team has developed a comprehensive solution to increase the feasibility and maximize the value of RWE programs for targeted cancer therapies, including:

- **Access to linked biomarker & clinical data:** By partnering with leading oncology centers in Europe, this solution combines rich biomarker data with clinical Electronic Medical Record (EMR) data. It enables various types of RWE studies and enhances efficient site selection, expediting evidence generation and market access.
- **Retrospective testing of archival tissues:** Collaboration with biobanks and pathology labs allows for retrospective testing of archival tissue samples, broadening our understanding of novel and under-tested biomarkers. This efficient and cost-effective approach speeds up precision oncology studies.
- **Enabling AI-Powered precision oncology:** This solution streamlines access to imaging and pathology data for pharmaceutical partners. Whether for detecting oncology biomarkers from pathology images or discovering radiomics-based biomarkers, these data modalities are key for leveraging machine-learning technologies.

Our dedicated solution enables us to run effective RWE programs for targeted cancer therapies, even for novel and under-tested biomarkers, and helps our pharmaceutical partners get the most value from these programs.

Introduction: Real World Evidence (RWE) for precision oncology

Targeted therapies are instrumental in revolutionizing cancer treatment, as they provide personalized care to each patient. Products that necessitate biomarker testing account for approximately 60% of the oncology market share[1]. Remarkably, this segment is experiencing the highest growth rate within oncology, boasting a robust compound annual growth rate (CAGR) of 21%[1]. An examination of the oncology pipeline suggests that this trend is expected to persist in the future.

These targeted therapies inherently face a unique set of challenges when it comes to generating clinical evidence and market entry. This is where Real World Data (RWD) plays a crucial role. Drawing from our experience in assisting leading pharmaceutical companies, RWD offers numerous benefits in the development and commercialization of targeted oncology therapies.

These benefits include

Accelerating clinical development by utilizing real world control arms: Leveraging RWD as external control arms can accelerate pivotal clinical studies. This is particularly beneficial for targeted therapies, where patient numbers are inherently low. In the period of 2019-2021, 85% of FDA submissions (NDAs and BLAs) made use of RWD for therapeutic context, safety, effectiveness, or a combination thereof[2]. Similarly, RWD served as an external comparator for clinical efficacy verification in 17% of EMA submissions from 2016 to 2021[3].

Supporting market access by generating robust Real World Evidence: RWD is growing in importance to evidence generation for negotiations with payers and Health Technology Assessment (HTA) bodies. An IQVIA analysis showed RWD use in HTA report submissions increased from 6% in 2011 to 39% in 2021[4]. This role is particularly significant for targeted therapies, where market adoption is typically slower, making prospective observational studies a less than ideal solution for post-launch evidence generation.

Driving market penetration by promoting biomarker testing practices: RWE is instrumental in understanding and promoting biomarker testing practices, which are crucial for introducing targeted therapies to the market. We have witnessed large variations in adoption rates between cancer targeted therapies, mainly due to differences in testing practices. RWD studies are vital for engaging Key Opinion Leaders (KOLs) and increasing product awareness within the medical community.

Challenge: RWE with novel biomarkers

Utilizing RWD for targeted therapies is an imperative for innovative pharma companies, yet it is a task riddled with challenges. According to an IQVIA Oncology Dynamics report, cancer patients in the EU5 countries are tested for an average of one biomarker, predominantly in specific indications such as lung and breast cancer[5]. Despite the rapidly growing list of relevant biomarkers, the rate of clinical adoption is considerably slower, making it difficult for companies to generate RWE for their targeted drugs relying on biomarkers not commonly tested in routine clinical practice.

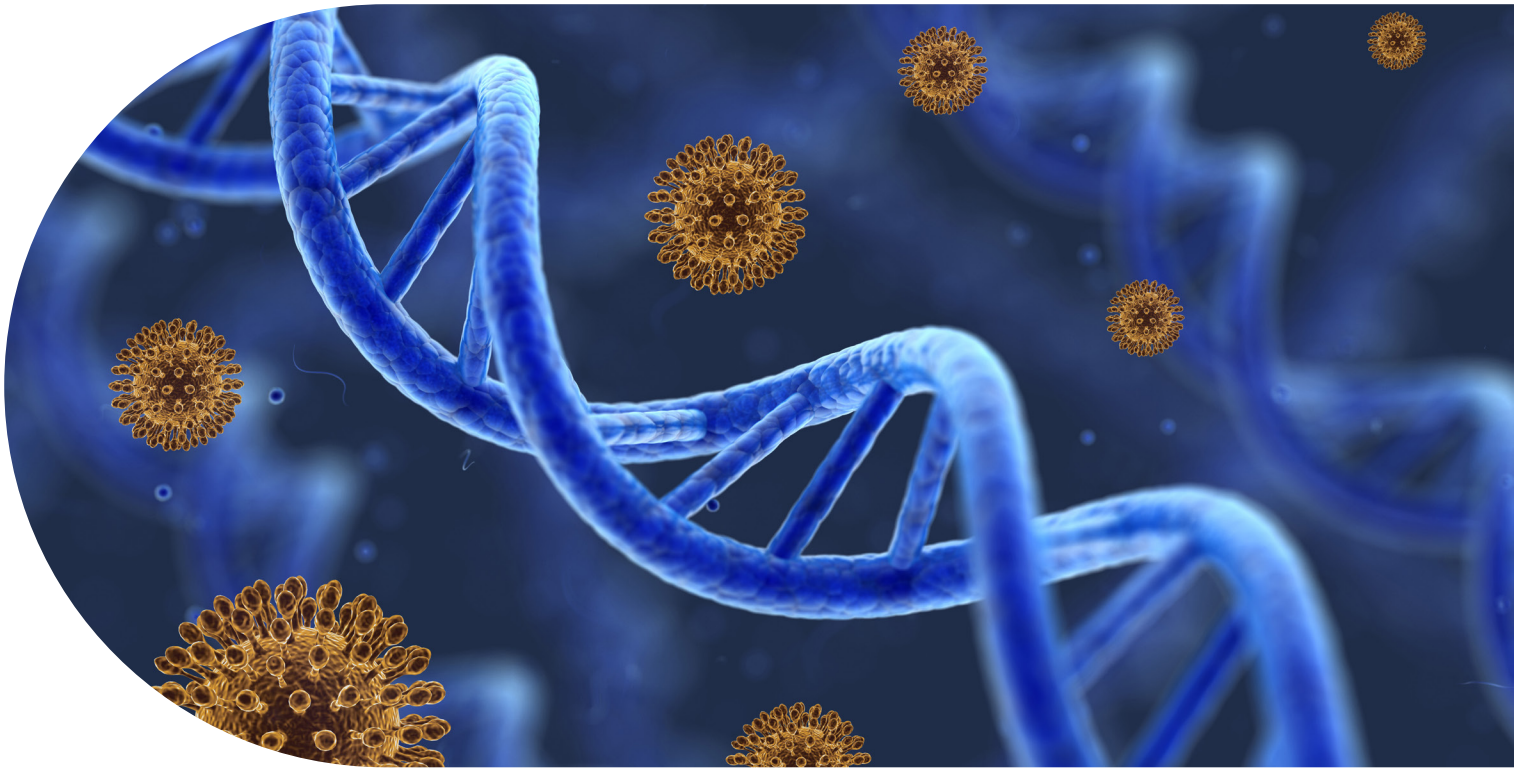
Several key challenges consistently emerge in this field

Variability in testing practices makes it difficult to choose the right sites: Across various indications, countries, and even within clinics in the same nation, inconsistent testing practices complicate the planning and execution of RWD studies, often affecting timelines and budgets. A recent example we encountered is the inconsistent testing of ESR1 in breast cancer patients, which is being tested routinely by cancer centers in France but not in Germany.

Slow adoption of novel biomarkers extends the timelines of RWD programs: The integration of novel cancer biomarkers into national clinical guidelines is a lengthy process, and once included, they're usually recommended for narrow indications only. For instance, despite its potential to alter treatment paradigms across multiple tumor types, HER2 is only recommended for patients with breast or gastric cancer[6].

Low prevalence of biomarkers make RWD studies extremely challenging: Some impactful biomarkers exhibit low prevalence, making it difficult to conduct adequately powered studies that will gain acceptance from regulators and payers. An example of this is the ROS1 rearrangement, a relevant lung cancer biomarker, which is only prevalent in 1-2% of patients[7].

The given challenges highlight the need for developing innovative solutions that integrates biomarker data into clinical data to enable robust and efficient RWE generation for targeted therapies.



Solution 1: Access to linked biomarker & clinical data

The Oncology Evidence Network (OEN) has forged significant partnerships with over 30 elite oncology centers in Europe, leveraging this network to access comprehensive biomarker and clinical EMR data. This collaboration has enabled us to generate robust real-world data to support some of the innovative therapies in oncology. OEN continuously evaluates new biomarker testing approaches, allowing for the development of targeted RWD programs and the support of pharmaceutical companies in developing and launching their innovative targeted treatments.

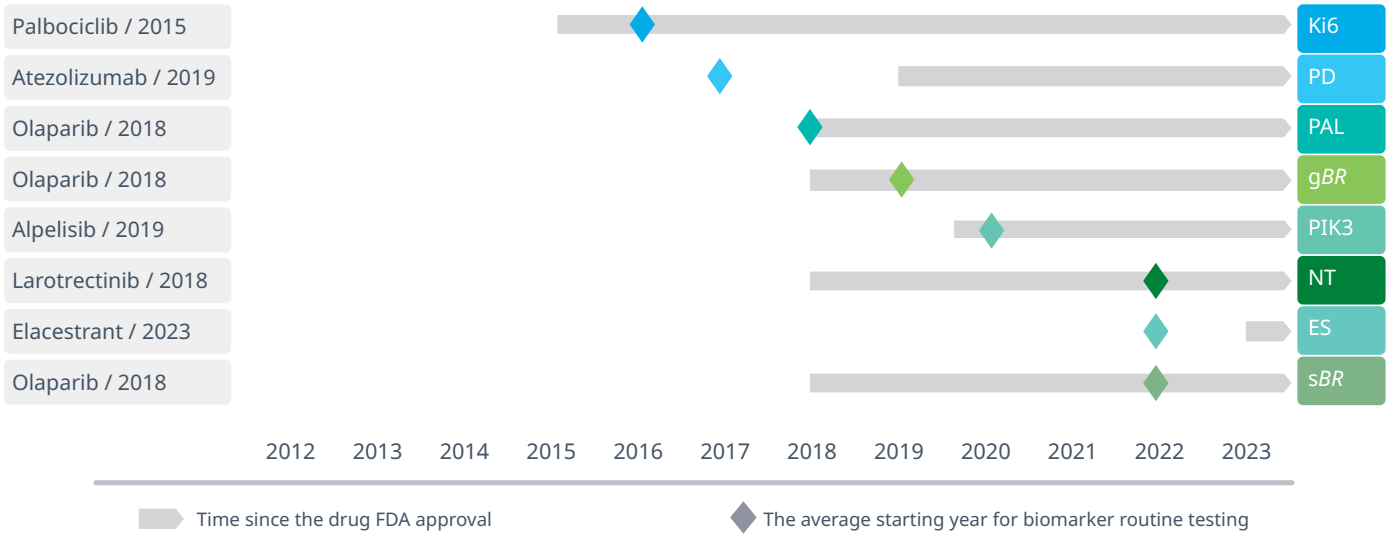
Highlighting the multifaceted advantages of our network:

Combining biomarker and EMR data to perform all types of RWD studies: Our comprehensive solution enables us to perform a wide range of RWE-studies in all oncology indications, including studies that have granular inclusion criteria or require testing for novel biomarkers.

Effective evidence generation with efficient site selection and timelines: Our solution aids in the structuring of efficient RWD programs and in the selection of the best clinical sites, hence expediting the evidence generation and market access for targeted cancer therapies.

Partnership with the leading oncology centers across europe: Our partnership with expert cancer centers and academic hospitals ensures that our data collection methods and investigative studies meet the highest standards of clinical research.

The Start of the routine testing for selected biomarkers in breast cancer at a sample OEN site, combined with the timeline of the FDA approval for the biomarker-relevant therapies



Sources: IQVIA Oncology Evidence Network – OEN

Solution 2: Retrospective testing of archival tissues

The collaboration with our extended partner network, which includes leading biobanks, pathology providers, and labs allow us to offer retrospective testing of archival tissue samples. By integrating this testing with the existing Real World clinical data, we can uncover new insights into novel and under-tested biomarkers. This approach not only broadens our understanding of these biomarkers but also provides critical evidence for the effectiveness of targeted therapies.

This unique solution means that many biomarker-driven studies that were once only feasible prospectively, can now be conducted retrospectively, saving years in the study timeline.

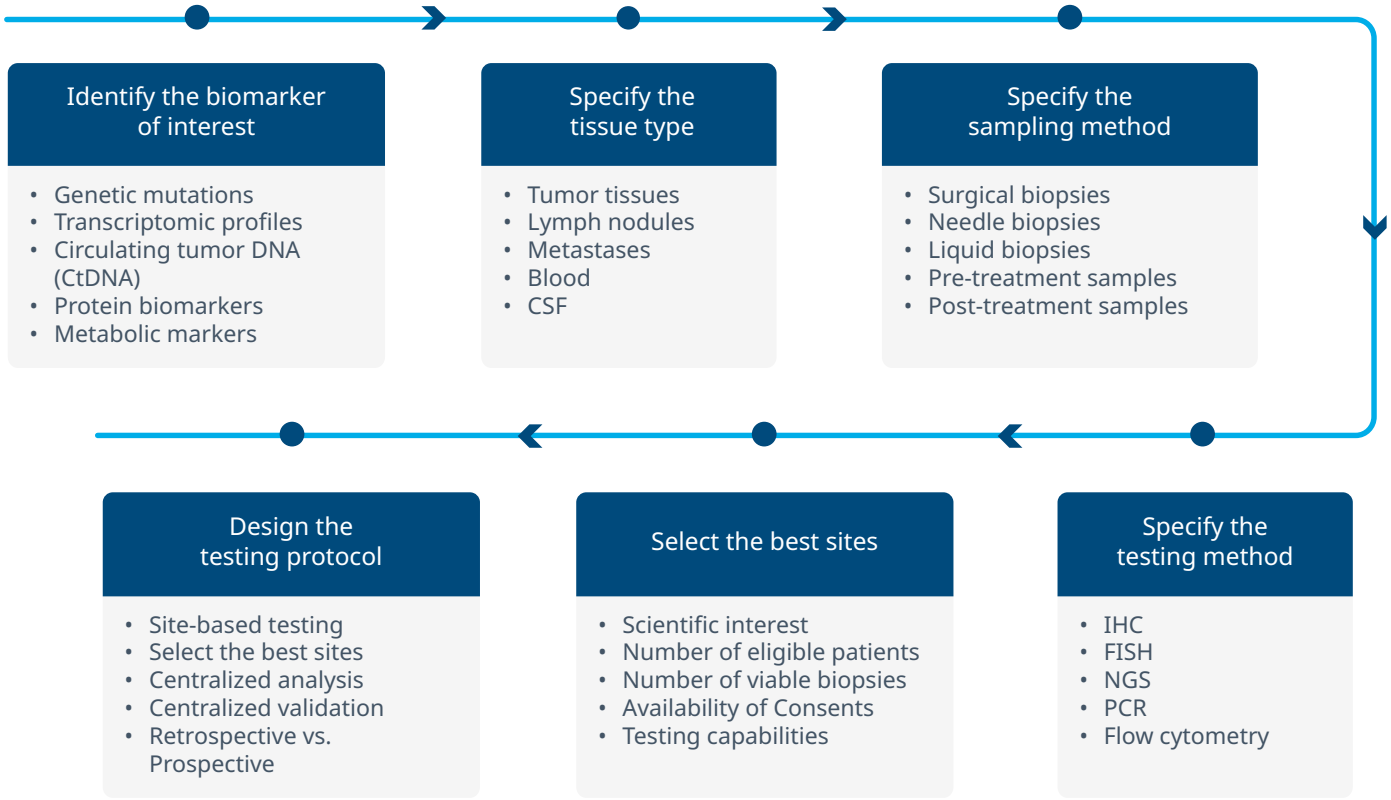
The advantages of our solution include

Cutting-edge retrospective testing for novel and under-tested biomarkers: Our approach enables us to perform retrospective testing within feasible budgets and tightly controlled timelines, making it a more cost-effective solution compared to traditional prospective studies.

Strategic site selection based on biomarker testing capabilities: Our unique network allows us to hand-pick oncology sites with extensive tissue sample repositories and broad consents, ensuring representation of the relevant patient population.

Robust Real World Evidence enabled by biomarker testing: By enabling access to archival tissue sample to be tested retrospectively, we can perform statistically powered precision oncology studies. This includes studies that were deemed unfeasible before, due to the lack of routine biomarker testing.

The process of planning and performing retrospective testing of archival biopsies



Sources: IQVIA Oncology Evidence Network – OEN

Solution 3: Enabling AI-Powered precision oncology

Artificial intelligence (AI) is revolutionizing several aspects of developing new treatments, diagnosing and treating diseases. Precision oncology is set to benefit from these technological advancements, particularly via AI-enabled detection of oncology biomarkers starting from pathology whole slide images (WSI). Additionally, we are seeing advances in radiomics-based biomarkers that could be used in diagnosing, treating, and monitoring cancer patients.

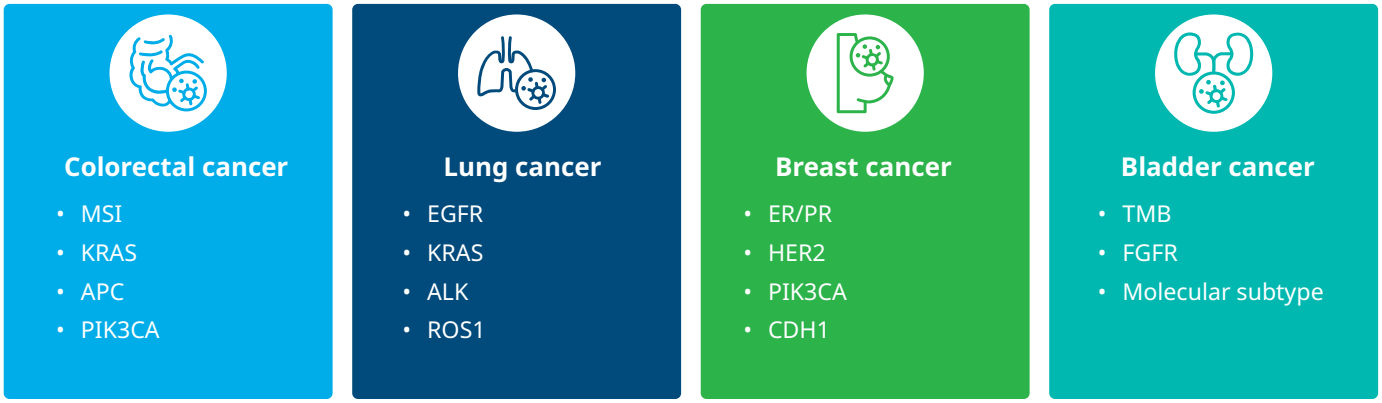
With an eye on the future, we are working with our oncology network to streamline the access to imaging data for pharma partners to leverage new technologies as a part of their Real World strategy.

The advantages of our solution include

Enabling access to imaging and pathology data: We are working continuously with our partner clinics to enable access to new data modalities, with a focus on radiology imaging and digital pathology slides. Combined with rich clinical data, imaging data can be leveraged to provide more insights and discover new diagnostic and stratification methods.

Leveraging innovative technologies for more insights: By collaborating with internal and external technology partners, we can provide our pharma partners with end-to-end solutions that enable them to develop machine-learning algorithms for disease diagnostics and biomarker detection, maximizing the value of their real world data projects.

Biomarkers identified in images of H&E-stained pathology slide through deep learning algorithms



Sources: [8] [9] [10] [11] [12] [13] [14] [15]

Conclusion

As the realm of precision oncology evolves, embracing RWE is no longer an option but a necessity. The path forward mandates a collective action where pharmaceutical companies, oncology centers, and stakeholders at large, rally behind data-driven, patient-centric solutions like ours. The promise of delivering personalized, effective cancer treatment is within reach. Now is the time to act, to foster collaborations, and to invest in RWE programs that will define the future of cancer care.

Leveraging our extensive experience in Real World Evidence (RWE) studies with esteemed pharma companies and top cancer centers in Europe, we have crafted a comprehensive solution addressing the rising demand for RWE in cancer targeted therapies across various use cases. Our innovative approach marries Real World Data with biomarkers, even those novel or under-tested, expediting clinical development, market access, and penetration for targeted therapies.

Interested in learning about our Oncology Evidence Network (OEN) and offering?

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Tarek is a senior consultant at the oncology evidence network (OEN). He leads the partnerships with the oncology clinics in Germany and Austria and is developing the precision oncology offering.

Tarek is a medical doctor by training. He has gained experience in clinical trials, venture capital, and health tech.

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