CASE STUDY

From RUO to Trial-Ready in Six Months

Accelerating an FGFR NGS Patient-Selection Assay for a Global Phase 3 solid tumor Cancer Trial

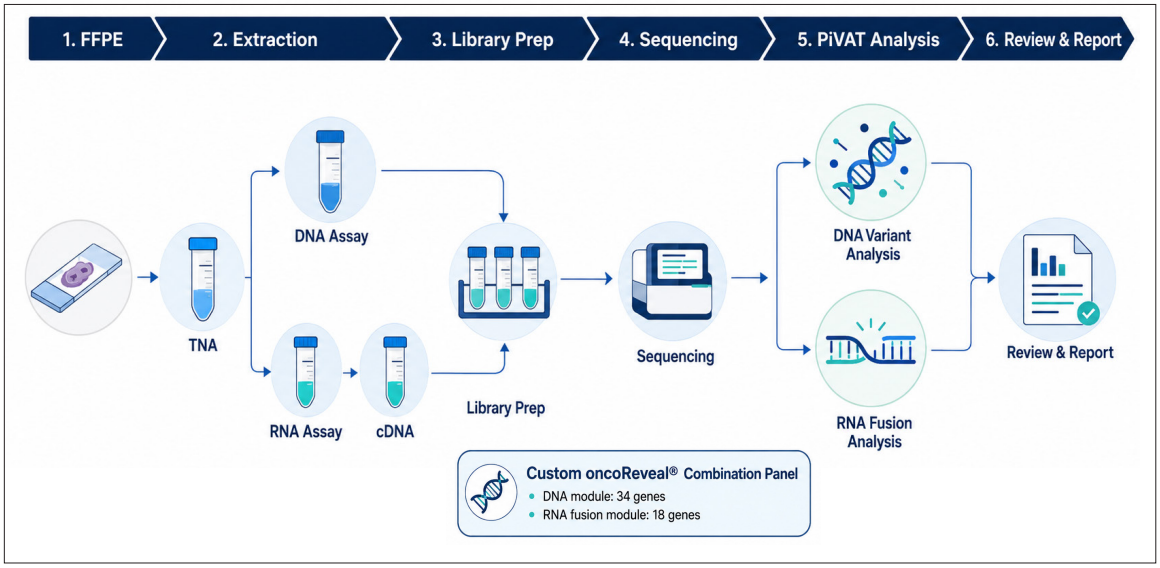
When a global biopharmaceutical company needed to replace an obsolete qPCR-based patient-selection assay for a Phase 3 cancer trial, speed was essential, but speed alone was not sufficient.

The new assay needed to detect a defined set of clinically relevant FGFR mutations and fusions starting from formalin-fixed, paraffin-embedded tissue. No existing, commercially available panel provided the complete coverage required by the clinical development program. At the same time, the assay had to progress rapidly from a research-use-only to an investigational-use-only solution, undergo clinical trial assay validation, and support the regulatory documentation required to initiate patient testing.

CellCarta and Pillar Biosciences established an integrated development model that brought assay design, kit development, analytical validation, laboratory implementation, regulatory support, and clinical execution together as parallel—not sequential—workstreams.

Using the oncoReveal® Essential and Fusion next-generation sequencing panels as the foundation, the Pillar Biosciences team developed a combined NGS solution in one kit with the required variant coverage and optimized it for use with FFPE. CellCarta completed the clinical trial assay validation and prepared the laboratory workflow for patient-selection testing, while the Pillar Biosciences project team completed the required studies to support the Investigational Device Exemption and associated PSA submission requirements.

The result was a trial-ready patient-selection assay delivered in less than 6 months rather than the standard 10-12 months, enabling the clinical study to proceed according to its planned first-patient enrollment timeline.



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The Challenge: replacing a legacy assay without delaying a pivotal trial.

Biomarker-driven oncology trials depend on the availability of a reliable patient-selection assay. When that assay is unavailable, obsolete, or not sufficiently comprehensive, the diagnostic workstream can become a critical-path risk for the entire therapeutic development program.

For a Phase 3 program in FGFR-driven solid tumor cancer, the sponsor had previously relied on a qPCR-based assay. That assay was no longer a viable solution for the planned study, creating an urgent need for a replacement.

The requirements extended beyond identifying a technically capable NGS platform. The new solution needed to:

- Detect the complete set of FGFR mutations and fusions required by the clinical protocol.
- Perform reliably with FFPE tumor tissue.
- Support prospective patient identification and enrollment.
- Transition from an RUO-labeled kit to an IUO configuration suitable for use in the clinical trial.
- Be validated as a clinical trial assay under an appropriate quality framework.
- Generate the data and documentation needed to support IDE and associated PSA submissions.
- Be transferred, implemented, and operationally ready within an aggressive Phase 3 timeline.

No existing panel met all these requirements without modification. A conventional sequential development process—panel selection, customization, kit conversion, validation, regulatory preparation, laboratory transfer, and trial implementation—would have created substantial schedule risk.

The project therefore required a different operating model.

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An integrated development model enabled by a strategic partnership.

CellCarta and Pillar Biosciences formed a coordinated project team with clearly defined but highly interconnected responsibilities.

Pillar contributed its targeted NGS technology and kit-development capabilities. CellCarta contributed its clinical laboratory, assay validation, regulatory support, and global clinical trial operational expertise.

Rather than handing the assay from one organization to the other after development was complete, the teams worked concurrently. Decisions made during panel configuration were evaluated against downstream validation, regulatory, sample-processing, reporting, and clinical implementation requirements.

This joint model enabled the assay to be designed not simply as a technically functional panel, but as a complete patient-selection solution capable of supporting a late-stage clinical trial.

The program incorporated 5 parallel workstreams delivering speed while maintaining scientific rigor:

1. TARGET COVERAGE AND PANEL CONFIGURATION.

The teams assessed the sponsor's required FGFR alterations against the coverage available through Pillar's existing oncoReveal® Essential and Fusion assays. A combined NGS approach was developed to provide the required coverage across relevant mutation and fusion classes.

Design decisions incorporated both the biological requirements of the clinical program and the practical constraints associated with FFPE tumor specimens.

2. RUO-TO-IUO TRANSITION.

Pillar advanced the assay configuration from its RUO starting point toward an IUO-labeled solution appropriate for investigational clinical use.

This work proceeded in parallel with CellCarta's validation activities, reducing the delay that can occur when kit development and laboratory validation are treated as separate, consecutive phases.

3. CLINICAL TRIAL ASSAY VALIDATION.

CellCarta developed and executed the validation strategy required to establish the assay as a fit-for-purpose clinical trial assay for prospective patient selection.

The validation program addressed the assay workflow from FFPE specimen receipt through nucleic acid processing, sequencing, data analysis, variant interpretation, quality control, and reporting.

Close coordination with Pillar allowed technical observations identified during validation to be addressed rapidly and incorporated into the evolving assay and kit configuration.

4. REGULATORY DOCUMENTATION AND SUBMISSION ENABLEMENT.

Because the assay would influence patient eligibility for an interventional oncology trial, regulatory planning was integrated into the project from the outset.

The teams generated the technical and validation documentation necessary to enable the sponsor's IDE and associated PSA submissions. Aligning the development, validation, and regulatory workstreams reduced the risk of late-stage documentation gaps or inconsistencies between the assay used in the trial and the assay described in the regulatory package.

5. LABORATORY TRANSFER AND CLINICAL EXECUTION.

CellCarta established the controlled laboratory workflow, trained personnel, implemented quality controls, and prepared the operational infrastructure needed to support patient testing.

The implementation plan addressed not only analytical performance, but also the turnaround time, specimen management, data review, reporting, and issue-escalation processes necessary for a global\ Phase 3 study.

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Delivering multiple critical-path activities concurrently.

The principal advantage of the collaboration was not a single technical breakthrough. It was the ability to manage interdependent activities as one integrated program.

In traditional assay-development models, each stage may begin only after the prior stage has been substantially completed. That approach can be manageable in early research but becomes increasingly risky when a patient-selection assay sits on the critical path to Phase 3 trial activation.

For this program, CellCarta and Pillar used overlapping workstreams and joint governance to compress the timeline while maintaining appropriate quality and documentation standards.

Panel design informed validation planning. Validation findings informed kit development. Regulatory requirements informed documentation from the beginning. Laboratory operations were prepared while the assay was being finalized rather than after development had concluded.

This approach minimized handoffs, shortened decision cycles, and allowed technical and operational risks to be identified earlier.

The Outcome: a trial-ready FGFR NGS solution in approximately six months.

In less than 6 months of project initiation, the collaboration delivered an operational patient-selection solution for the Phase 3 solid tumor cancer program.

Key outcomes included:

- Development of an NGS-based solution covering the required FGFR mutations and fusions.
- Optimization and validation of the assay for FFPE tumor tissue.
- Advancement of the underlying kit from an RUO-labeled configuration toward IUO clinical use.
- Completion of the clinical trial assay validation.
- Enablement of the required IDE and associated PSA submissions.
- Transfer and operationalization of the assay for prospective patient testing.
- Readiness to support the sponsor's planned first-patient enrollment timeline.

By integrating the technology-provider and clinical-laboratory workstreams, the program avoided the extended timelines and fragmented accountability that can arise when assay development, validation, regulatory support, and trial deployment are managed by separate organizations.

Lessons for biomarker-driven clinical development.

This case demonstrates several principles that can help sponsors reduce diagnostic-related risk in late-stage oncology programs.

Start with the complete clinical and regulatory endpoint.

Assay development should begin with the intended clinical use, specimen type, required variant coverage, trial design, regulatory pathway, and operational environment—not simply with the selection of a technology.

Run development and validation in parallel where possible.

Early coordination between the kit developer and the validating clinical laboratory allows assay-design decisions to anticipate downstream validation and implementation requirements.

Integrate regulatory planning from the outset.

When an investigational assay will determine patient eligibility, the documentation required for regulatory submissions should be built into the development plan rather than assembled retrospectively.

Treat operational readiness as part of assay development.

A technically successful assay does not support a clinical trial until it can be performed reproducibly, within the required turnaround time, under controlled laboratory processes, and at the necessary operational scale.

Establish joint governance and rapid decision-making.

Compressed timelines require clear ownership, defined escalation pathways, frequent technical communication, and rapid resolution of issues across organizations.

CellCarta accelerated an FGFR NGS patient-selection assay for a global Phase 3 solid tumor cancer trial, bringing it from RUO to trial-ready in less than 6 months

Biomarker-driven clinical trials can be delayed when the required patient-selection assay does not exist in a trial-ready form. This risk is particularly acute in late-stage development, where diagnostic timelines must align with fixed regulatory and patient-enrollment milestones.

Through an integrated development model, CellCarta and Pillar Biosciences transformed an existing RUO NGS foundation into an IUO, validated, and operational patient-selection solution for a global Phase 3 solid tumor cancer program in approximately five to six months.

The collaboration illustrates how coordinated assay development, clinical validation, regulatory enablement, and laboratory implementation can compress timelines without treating quality or regulatory readiness as downstream activities.

For therapeutic developers, the broader lesson is clear: engaging the technology provider and clinical laboratory as an integrated team can turn a potential critical-path obstacle into an accelerated route to patient enrollment.

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ABOUT CELLCARTA

CellCarta is a global CRO supporting biopharma with reliable, regulatory-ready biomarker data from translational research to CDx. With fully owned and harmonized laboratory operations across North America, Europe, Australia, and China, CellCarta delivers integrated capabilities spanning immunology, histopathology, proteomics, and genomics, supported by comprehensive sample management and logistics solutions.

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