Rarefied Thinking in Rare Disease Research

Specialized Insights Across Clinical Development

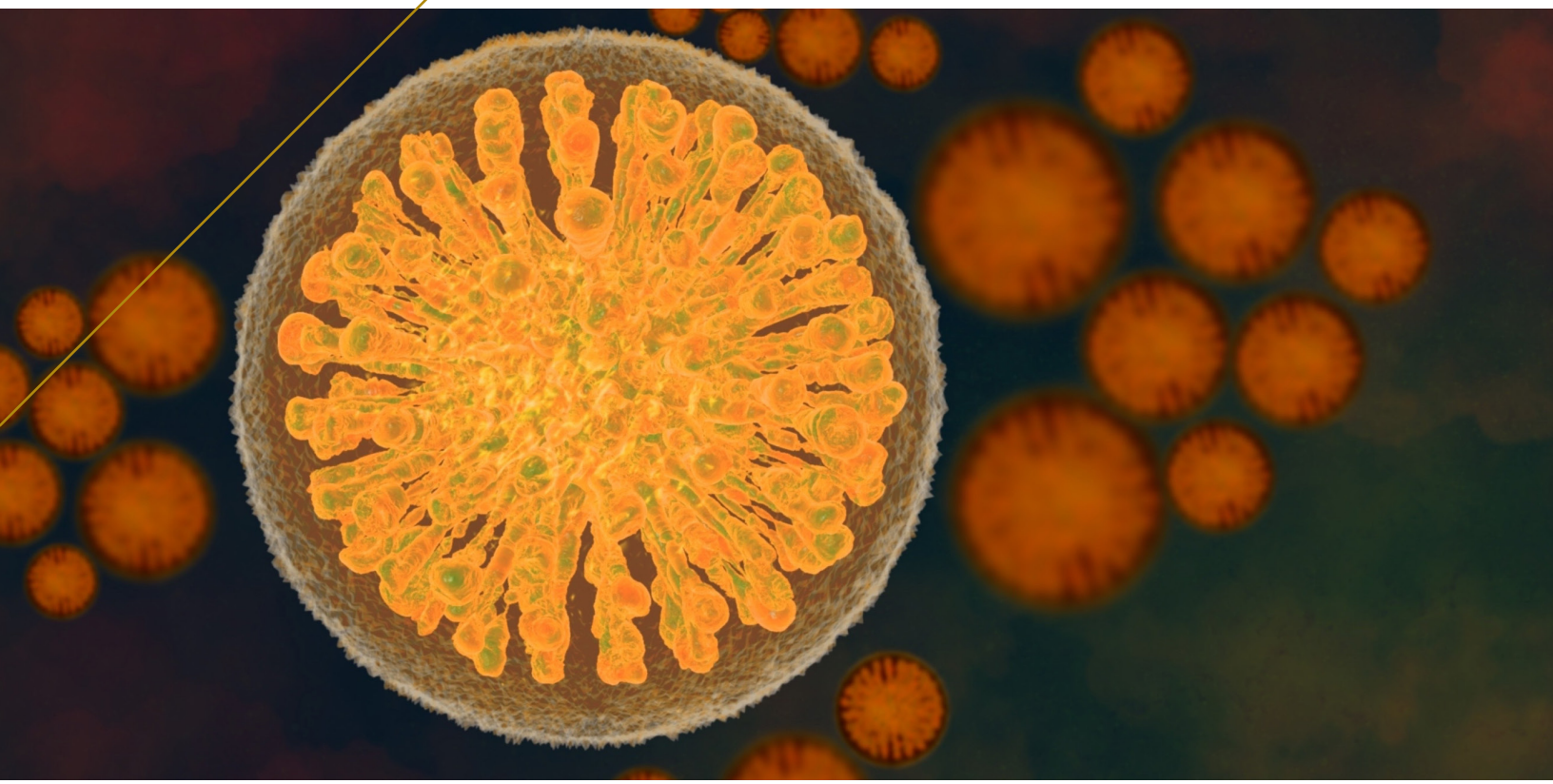
PRECISION for medicine

RAREFIED THINKING

When every patient matters, rare study challenges require rarefied solutions

Rarefied Thinking

INSIDE



NEARLY 200 PROJECTS ACROSS ~100 RARE AND ULTRA-RARE INDICATIONS GROUND OUR UNDERSTANDING AND APPROACH

Rare disease research is a high-stakes, high-complexity endeavor. With a significant percentage of global biotech and pharmaceutical companies pursuing therapies in the rare disease space, competition is fierce. Success in this space will depend upon a myriad of factors across the development and commercialization lifecycle, from study design and recruitment to sample management and manufacturing. Tailored, fit-for-purpose solutions can help to address the unique challenges associated with studying patient populations that are inherently limited, often heterogeneous, and typically geographically dispersed.

Rare disease research often breaks new ground and includes innovative approaches to clinical development and commercialization to satisfy a significant unmet need. Addressing the unique challenges of each indication and study calls for diverse perspectives, novel solutions, creativity, and agility—what Precision calls, Rarefied Thinking.

There is an urgency and responsibility to maximize the meaningful data extracted from each patient interaction and sample. This eBook offers insights on overcoming specific challenges encountered during rare disease development and commercialization.

We believe the key learnings from our experience can help you better navigate the journey ahead, allowing you to one day bring hope to those with little.

KEY CONSIDERATIONS WHEN CONDUCTING A "MAKE VS BUY" ANALYSIS FOR PRODUCT MANUFACTURING

Contrary to where it falls on the development timeline, manufacturing is one of the earliest obstacles a rare disease therapy faces. The technology required to scale up from the laboratory needs to be tailored for high value at low volume. For organizations pursuing cell or gene therapies for a targeted disease, there are additional layers of complexity that impact planning and development.

Long before an investigational product has achieved regulatory approval, sponsors need a solution for scaling up the manufacturing process from the lab to a Good Manufacturing Practice (GMP) facility. This important strategic process makes delivering life-saving therapies to the patients cost-effective and enables the broadest possible access.

Should You Build Your Own GMP Facility or Use a CDMO?

There are two manufacturing options companies are presented with as they prepare to bring new products to market:

1. "Make" – where capital is invested to design and construct an in-house GMP facility
2. "Buy" – where scale-up and commercial production are contracted out to a contract development and manufacturing organization (CDMO)

As leadership, investors, and technical operations advisors begin to plan for clinical and/or commercial production and to decide between "Make vs Buy," there are several key topics that must be addressed:

- Patient population
- Dose per patient
- Scale-up timeline and yield projections
- Product pipeline
- Production throughput (both at regulatory approval and throughout the product lifecycle)

Accurately assessing and answering these areas of inquiry requires cross-functional collaboration among key stakeholders and expert advisors as well as R&D, regulatory, and market research teams. Here, the ultimate goal is comparing the cost per dose and ROI for each manufacturing option. R&D is responsible for developing a robust GMP process that can be used to:

- Estimate production costs including raw materials, equipment, and facility space
- Compare the estimated cost and timeframe of manufacturing in-house versus outsourcing production to a CDMO

Consider Lead Time and Capital Requirements

Time is of the essence when bringing a product to market, and building an in-house GMP facility may require significant upfront capital. In some cases, it makes sense to build a phase-appropriate manufacturing facility if a suitable CDMO partner or an ideal production slot is not available.

Considerations for Manufacturing In-House

If your company is using a unique and proprietary manufacturing technology to develop your product, outsourcing may not be feasible, and construction of a facility may be required.

Potential advantages of manufacturing in-house include:

- The ability to own the production process from start to finish, offering greater control and demonstrating the ability to execute and create a pipeline of products
- Intellectual property protection, greater flexibility, and lower long-term costs, which is attractive to investors
- Potential to differentiate from competitors and increase company valuation, especially in the cell and gene therapy space

Considerations for Outsourcing Manufacturing

If your company does not have sufficient capital or technical capability to build out an in-house manufacturing capability, partnering with a CDMO that has relevant product and indication experience may be the only option. Special consideration is required for outsourcing the manufacturing of cell and gene therapy products, which involves complex processes and logistics and can be more challenging to scale. Planning for commercialization in these early stages is difficult in the absence of knowledge about exactly what will be required to scale, what launch quantities will look like, or what market penetration will be.

Key Takeaway

Manufacturing decisions for rare disease products may need to be made much earlier in the development process than with traditional therapies due to expedited approval pathways. Significant planning is required for determining how to scale benchtop manufacturing to support clinical trials and, eventually, commercialization. Starting as early as possible and adopting a strategic approach to decision-making will help your organization select the manufacturing option that is most appropriate for your company and your specific product.

PROJECT FARMA
A PRECISION FOR MEDICINE COMPANY

Project Farma is the manufacturing-focused arm of Precision for Medicine's end-to-end service offering, specializing in rare diseases and able to provide strategic manufacturing guidance on advanced therapies and next-generation medicines.

INTEGRATING HEALTH ECONOMICS AND OUTCOMES RESEARCH TO ENHANCE RARE CLINICAL PROGRAM DEVELOPMENT

The early involvement of health economic and outcomes research (HEOR) can help to mitigate some of the notable issues standing in the way of rare disease research, including:

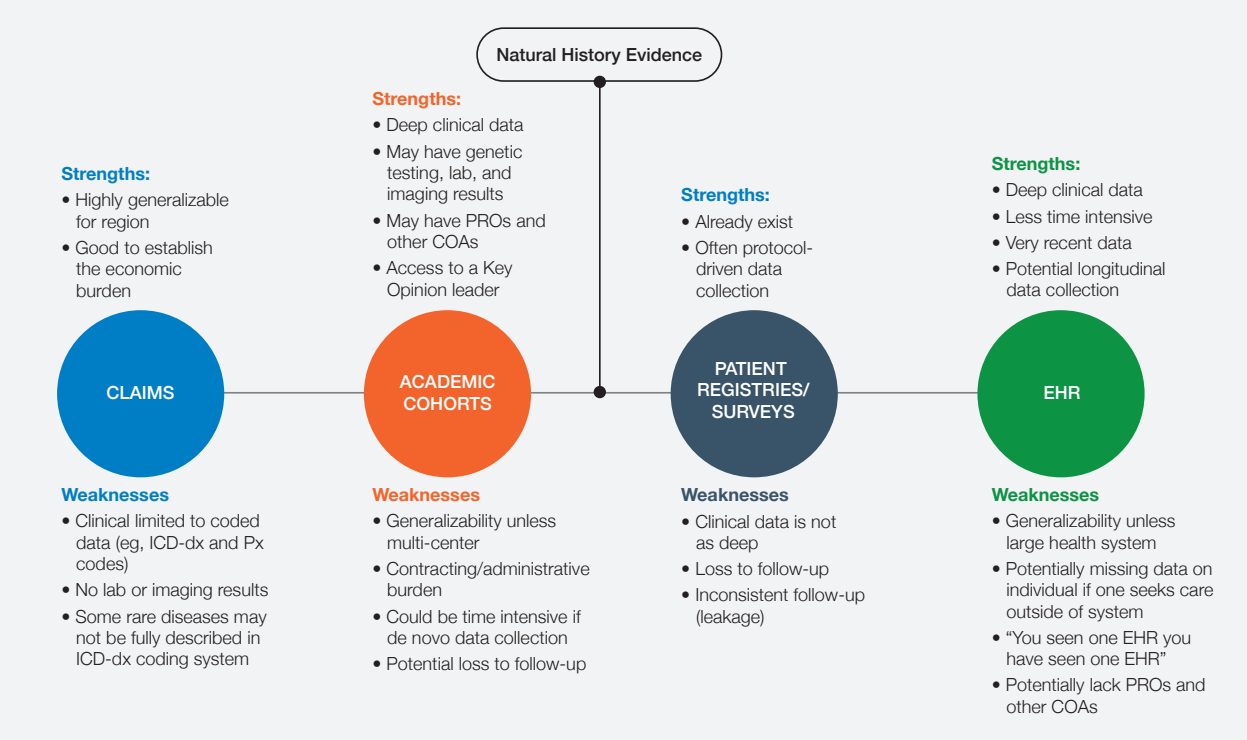
- Limited understanding of the natural history of the disease
- Difficulty in identifying the right study participants
- Lack of existing models for defining or demonstrating value

> *"When you deeply understand the disease, you can more easily see the person. That's what we want to matter; we want to be seen."*[1]
>
> —Kyle Bryant
> Spokesperson, Friedreich's Ataxia Research Alliance (FARA)

Performing Natural History Studies to Inform Clinical Trial Design

As natural history data are often limited for rare diseases, these studies and analyses play an important role in drug and clinical development. Natural history studies can be conducted prospectively through primary data collection or retrospectively using various real-world data (RWD) sources (see Figure 1).

Figure 1 | **RWD Sources: Strengths and Weaknesses**



Natural History Studies Can Be Used for:

1. **Gathering information on disease subtypes**, including rate and patterns of progression, which can be used to inform the target study population and trial duration.
2. **Validating or refining outcome assessments** to gain insight into performance, including minimal clinically important differences.
3. **Developing or validating biomarkers**, which can be used to prognosticate disease course, predict treatment course, or guide patient selection in clinical trials.
4. **Serving as external controls for other clinical trials** where having a control group may be unethical due to the severity and life-threatening nature of the disease or it may be logistically unfeasible because of small sample size.[2]

Using Real-world Evidence to Identify Patients for Clinical Trial Recruitment

To address issues with recruitment, HEOR researchers commonly use datasets, such as open-source health insurance claims, to assist in locating clusters of individuals with a specific rare disease. Open-source health insurance claims are those that have been transmitted from a provider but have not yet been transmitted to the insurer. Of note, open-source health insurance claims are accessible in the United States but may not be available in other countries. These types of claims are valuable because they capture claims for all insurers and include a provider ID so that individuals with a rare disease can be tracked to a specific hospital or provider. Therefore, analyses can be performed to identify individuals with a suspected rare disorder and their providers to isolate clusters of high-throughput clinicians and centers for specific rare diseases to assist in clinical trial recruitment.

Developing Conceptual Early Health Economic Models to Influence Trial Design and Evidence Strategy

Pharmaceutical companies routinely develop health economic models, such as cost-effectiveness and budget impact models, for their assets. These models—which are based on results from pivotal trials and assess the direct and indirect costs and consequences of a new therapy—are often required by payers and health technology appraisal organizations (HTAs) to ensure access and reimbursement.

Since the early 2000s, many developers have used early health economic models as a best practice to inform clinical trial design to align with payer expectations and HTA assessments. Unlike HTA models, early health economic models are developed before the manufacturer has robust data on efficacy, safety, and other outcomes from clinical trials. The value of these early health economic models is their ability to demonstrate which clinical outcomes will drive value and highlight useful data additions to the clinical trial, such as quality-of-life assessments, which are needed to support utility measurement or additional outcome measures important to payers and HTAs.[3]

Integrating HEOR into early-stage development can profoundly impact your clinical development plan and strategy while incorporating much-needed real-world data.

Parting Thoughts

Currently, the trend is to begin HEOR activities earlier in the clinical program, especially for rare diseases. As HEOR tools can provide deeper insight into the rare disease and the individuals living with it, they can profoundly impact the development program thought process, allowing patients to be seen and their experiences infused into how we design and conduct trials.

References:

1. Kyle Bryant. "Meet Kyle Bryant." YouTube. Accessed February 2, 2023. https://www.youtube.com/watch?v=82x1m-NWODM
2. Liu J, Barrett JS, Leonard ET, et al. Natural history and real-world data in rare diseases: applications, limitations, and future perspectives. *J Clin Pharmacol*. 2022;62(suppl 2):S38–S55.
3. Jensen I, Cyr P. Early health economic models in gene and cell therapy to inform clinical trial design and optimize commercialization efforts. Pharmalive.com. December 6, 2022. Accessed December 29, 2022. https://www.pharmalive.com/early-health-economic-models-in-gene-and-cell-therapy-to-inform-clinical-trial-design-and-optimize-commercialization-efforts/

AWARENESS AND ENROLLMENT: COLLABORATING WITH PATIENT COMMUNITIES AND ADVOCACY GROUPS

There are more than 10,000 identified rare diseases impacting an estimated 400 million people globally.[1]

For rare indications, sponsors and CROs often scour the globe to find the right patients. While there is no one-size-fits-all approach to identifying, recruiting, and engaging meaningfully with potential study participants, there are strategies that can support an on-time study start.

- Individuals with rare diseases (and their caregivers) are motivated to join communities and seek online support
- Sponsors and CROs can collaborate with established groups and organizations to navigate existing community networks
- There may also be development and support opportunities as communities and patient advocacy groups vary widely in their capabilities

In this article, we share our tips and best practices for finding and approaching rare disease communities and advocacy groups, offering insight on how best to partner with them to maximize value and impact on your planned clinical trial.

Proven Strategies for Rare Advocacy Collaboration

Working directly with patient communities and advocacy groups can be a highly effective strategy for finding the targeted rare patients and making them aware of—and interested in—your clinical trial.

1.

Finding rare disease communities and advocacy groups

Identifying these communities and groups can be challenging, particularly when searching multiple regions, compensating for language barriers, and navigating locally preferred social media networks. Often, a multifaceted approach is required, and the following are some tips to help you get started:

- **Leverage established doctors and investigators**

Rare disease doctors typically have a national or global network that they access. Although this may be sufficient to meet your needs, it is more likely that you will need to further augment their network and rare population connections. Keep in mind that collaborating with doctors and hospitals can be helpful, but you may face communication challenges and need to work through getting the right people involved, as related to both sponsors and CROs.

- **Use advanced search engine techniques**

Search engines are vital resources for finding online communities, regardless of country. For more advanced Google search tips, please refer to this helpful article, which will help you to segment your search regionally.

- **Engage Facebook groups**

Facebook groups continue to attract active and engaged users. Reaching out to these groups can be a useful exercise, depending on the maturity of the target indication and the awareness surrounding it. Here is a helpful article for exploring Facebook groups

2.

Accounting for incidence and geography

One of the key strategies for working with patient advocacy groups is to map both the incidence rate and geographic implications of the disease. When selecting groups, understand their individual goals to help you identify those whose goals align with those of your clinical trial. To establish a successful communication channel, finding the right contact person—whether from the sponsor, CRO, or principal/local investigator—can make a tremendous difference in the overall success of the collaboration.

Mapping your incidence and geographic alighments to established and like-minded patient advocacy groups can create benefcial collaborations for all.

3.

Integrating advocacy groups into patient engagement plans

Advocacy groups can do more than bring patients to the study. They may also bring the sponsor or CRO closer to patients, allowing for a better understanding of the nature of the disease, its impact on all aspects of their lives, and any associated difficulties. As you search globally for advocacy groups, look for opportunities to connect with local, national, or regional communities as well. Integrating diverse groups into your patient outreach and engagement strategies will be critical to on-time enrollment success.

Not all sponsors or CROs spend enough time thoroughly investigating the communities associated with the targeted disease, nor do they fully leverage the potential of community and advocacy group collaboration.

Consider the Following Tips for Maximizing Your Outreach Potential:

- **Partner to understand the patient population**

This is particularly relevant if this is your first study of the indication of if you are planning a natural history study or launching a first-in-human clinical trial. Connect with several groups to ensure you understand the unique needs, preferences, and challenges of the population.

- **Collaborate on study awareness and enrollment**

Depending on the capabilities of the communities and advocacy groups, they may be able to support awareness and enrollment among their members using their existing communication channels, such as their website, newsletter, or social media pages.

Support Your Rare Community

These communities are safe and trusted spaces for people. It will be important to support any virtual or face-to-face gatherings normally conducted by the group, if possible, to help drive understanding, awareness, engagement, and trust.

The above strategies can help you tap into the connections that rare disease communities and advocacy groups already have with their patient populations and serve as an effective vehicle to drive awareness of your clinical trial. As a best practice, we suggest being receptive to all advocacy groups and keeping the door open to add new groups until study enrollment is complete. When masterfully executed, collaborations with patient advocacy groups can be a true catalyst for your trial's enrollment success.

Reference:

1. Global Genes. Rare Disease Facts. https://globalgenes.org/rare-disease-facts/. Accessed January 9, 2023

LEVERAGING GENETIC LABORATORIES FOR IDENTIFICATION OF PATIENTS WITH RARE DISEASES

‘Finding a needle in a haystack’ is a common analogy for clinical researchers looking for the right patients to enroll in a study, but in rare and ultra-rare indications, it can feel more like finding a snowflake in a snowstorm.

For certain indications, there are no patient communities of advocacy groups with which to engage. In these instances, collaborating with genetic laboratories can provide a much-needed boost to patient identification and recruitment.

Finding Genetic Laboratories for Rare Disease Studies

With the right laboratory, you can add the necessary testing capabilities for your study and leverage their data to support patient recruitment. Consider the following when beginning your search:

- Identify what type of lab would analyze the test in question—whether that be a genetic laboratory, specialty laboratory, or clinical laboratory—then, search for labs in the targeted enrollment regions
- To identify patients with genetic mutations, look for labs that already test for that specific gene. Key opinion leaders (KOLs) for rare diseases can often help you identify appropriate labs, giving you a starting point from which to expand your search, pending your study's patient count and country needs
- Once a lab is identified, contact them directly. You can inquire about the test in question via general inquiry forms on the laboratory's website or through its Business Development division

Genetic laboratories are likely already working or have worked with your targeted patient pool. Collaborating with them as part of your clinical trial or program's strategy can provide an additional pathway for patient and site identification.

Partnering with Genetic Laboratories for Patient Identification

After you have identified a few genetic laboratories in the countries or regions you are targeting and have confirmed they have the genetic test in question, it's time to engage. Because you are seeking assistance in identifying potential patients, you will want to ask about the following:

How often does the laboratory run the specific test? If they don't receive orders for this test often, it may not be a fruitful endeavor.

Can they confirm in their database the number of tests they have run for that target during the previous 6 to 12 months?

What is their process for raising awareness of clinical trials for a particular indication?

More specifically, can they provide a cover letter and an information sheet about the study to the ordering physician?

If they send a letter, is any additional follow-up conducted with the ordering physician? Discuss the parameters of this to ensure the best result possible from such outreach.

Finally, if the lab can support clinical trial awareness as described above, what is the cost for its assistance?

Mitigating a Key Challenge of Rare Disease Research

The use of existing databases or laboratory panels can help lead to the enrollment of previously or newly diagnosed patients. Finding genetic laboratories with the capability and willingness to support patient recruitment can help you sift through the haystack (or snowstorm) and find the patients you need for a successful study start.

Rare Disease CRO Services

With so much on the line, you want a CRO partner with the experience and expertise to stand shoulder-to-shoulder with you in finding out-of-the-box solutions. Precision for Medicine thrives amidst the nuances of the high-stakes rare disease landscape thanks to the depth and breadth of our experience across nearly 100 rare indications.

HOW CENTRAL LABS SOLVE FOR UNIQUE CHALLENGES IN RARE TRIALS

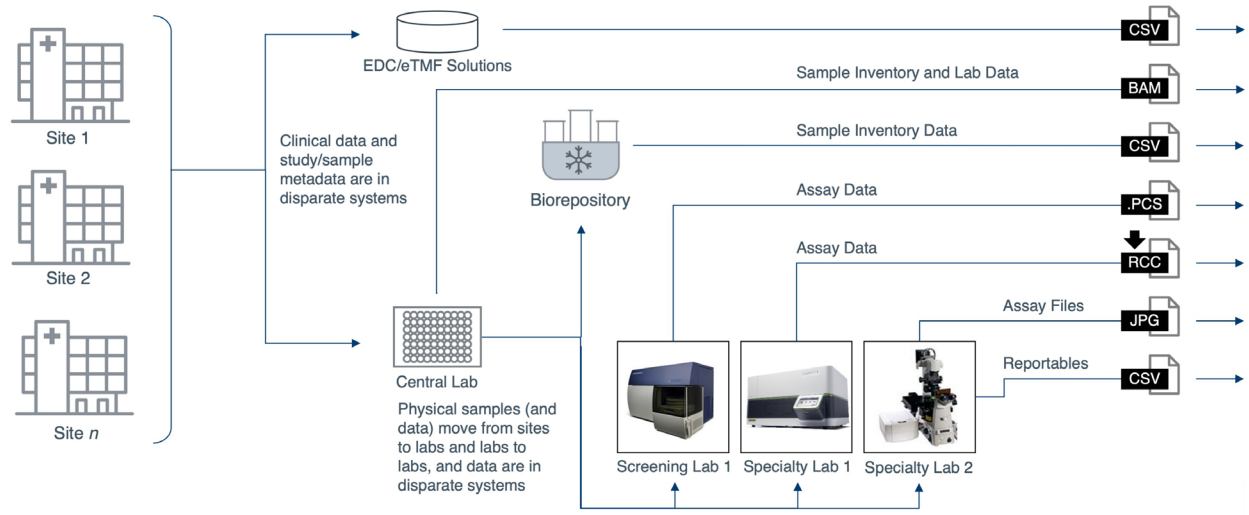
In today's rare disease landscape, where complex study designs are becoming more common, it is increasingly difficult to effectively coordinate all of the resources necessary to ensure success. Conducting rare disease clinical trials requires proactive planning and broad expertise not just in study execution and laboratory analysis but also in sample collection, processing, logistics, and data management. This has created a need for central lab services solutions that are tailored for rare disease trials.

Managing a Complex Ecosystem for Rare Disease Trials

Rare disease clinical trials often involve a proliferation of geographically dispersed sites, some of which are quite small but require the same level of management as larger institutions. These sites feed into a central lab, or sometimes multiple central labs, which then distribute samples to screening and specialty labs that perform the assays needed to generate data on the targets of interest. Each of these entities—every site and every central, screening, or specialty lab—represents a node in a complex ecosystem. Each node requires contracting, sample collection kits, chain of custody traceability, and data generation. All of this adds up to a substantial task of management, coordination, and data consolidation, beyond what is already required for study design, trial execution, regulatory filings, and other development activities.

Partnering with a central lab that has end-to-end services, mastery of logistics, and effective reporting technology is critical to rare disease sample management. When every patient and sample matters, leveraging a laboratory partner with rarefied solutions *truly matters*.

Figure 2 | **The Complex, Disconnected Ecosystem of Rare Disease Trials Leads to Sample and Data Chaos**

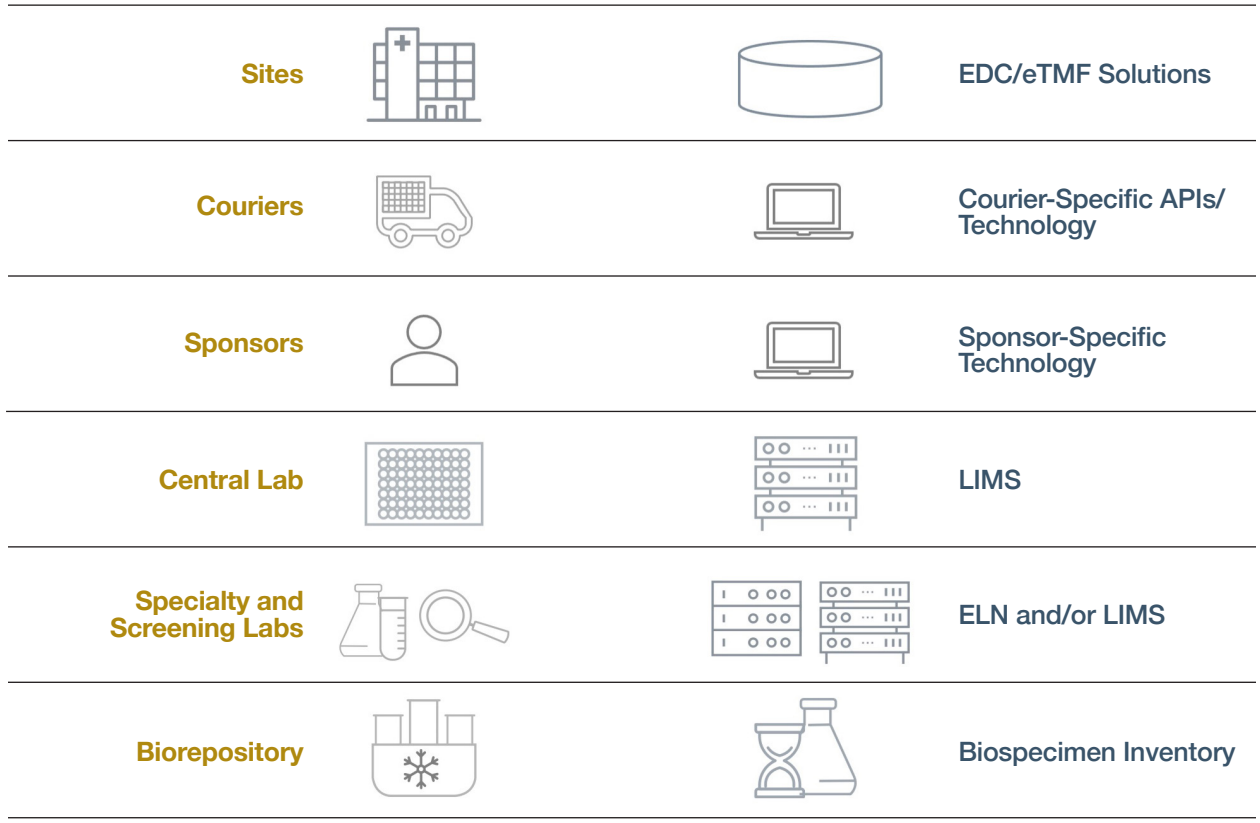


Compounding the complexity, the clinical trial ecosystem is often disconnected, creating operational and scientific challenges. Although clinical data is stored in an electronic data capture (EDC) system or electronic trial master file (eTMF), samples and their associated data move from sites to multiple labs in disparate systems and different formats. This increases the importance of accurate sample management for ensuring adherence to both the study protocol and the informed consent form (ICF).

Mapping Stakeholders in Rare Sample Management

When every patient is precious, so too is every sample collected. Sample management involves numerous stakeholders, each with their own system of generating and capturing data:

Figure 3 | **Sample Management Involves Multiple Stakeholders and Myriad Data Systems**



Sites need to have sample collection supplies on hand and are responsible for ensuring that samples are collected in accordance with the study protocol. Sample collection data are captured in an EDC or eTMF solution.

Couriers are responsible for transporting samples from sites to labs in a manner that is both visible and suitable for the sample requirements with respect to time, temperature, condition, and import/export regulations. Most couriers have their own application programming interface (API) or technology for data capture.

Central labs manage the primary responsibility of ensuring that chain of custody is maintained and appropriately documented from the sample collection site to each downstream control point. Data on sample type, de-identified participant information, sample conditions, labels, site, collection time, and more are collected in a laboratory information management system (LIMS). Depending on the study, central labs may also be responsible for:

- Conducting laboratory assessments
- Compiling test reports
- Contracting couriers for delivering and receiving kits and samples
- Producing kits, training sites on how to use them correctly, and managing kit expiry / inventory
- Processing samples
- Performing sample accessioning and logistics management
- Sending samples to screening and specialty labs for testing

Screening and specialty labs perform assays as per the study protocol and collect data in an electronic lab notebook (ELN) or LIMS.

Biorepositories may be needed for retaining samples with data capture in an inventory management system.

Sponsors may deploy technologies that integrate with any or all of the data streams above. Most often,

however, the first time a sponsor has visibility into the long chain of data related to clinical samples is upon arrival at the central lab.

Managing the multitude of disconnected technologies involved in conducting a rare disease clinical trial requires expertise, and central lab services play a critical role in ensuring seamless execution and access to critical data.

Streamlining Rare Sample Management

Integrating as many services as possible from a single provider can help streamline sample management and reduce sources of risk, delays, and costs. To manage all these complex movements, that single provider would need:

- Clinical trial expertise
- Courier management experience
- Longstanding international and domestic industry relationships
- Global, integrated networks of depots and storage facilities
- Knowledge of customs regulations and regulatory agency guidelines

Most importantly, that single provider would have to operate in accordance with a deep understanding of the importance of managing all clinical trial materials, equipment, and supplies under appropriate conditions and delivering them to the right location at the right time to support study success.

Another opportunity to streamline sample management is to use a virtual master sample inventory management system that consolidates all lab-related documentation in one place and offers centralized reporting via an intuitive user interface. This system should provide dashboards with key performance indicators (KPIs) and data summaries by site, subject, and sample type, allowing sponsors and CROs to identify patterns of noncompliance and focus on resolving discrepancies. Available on a web-based platform, this system enables more effective collaboration.

3 CRITICAL REPORTS FOR RARE DISEASE BIOSPECIMEN OPERATIONS

For clinical and biospecimen operations teams in rare disease drug development, tracking clinical samples is a critical but extremely time-consuming element of accurate trial execution. Teams are tasked with reporting sample collection status, consent status, and sample and derivative quality, but creating these reports often involves manually linking information from the electronic data capture (EDC) system, central labs, testing labs, and biorepositories to illuminate critical discrepancies or missed collections.

Below we identify three critical reports that sponsors and CROs should prioritize for effective biospecimen and biomarker operations. These reports highlighted below are most useful when they can be refreshed continuously throughout the course of a study and when all team members across an organization are accessing a centralized source of sample information.

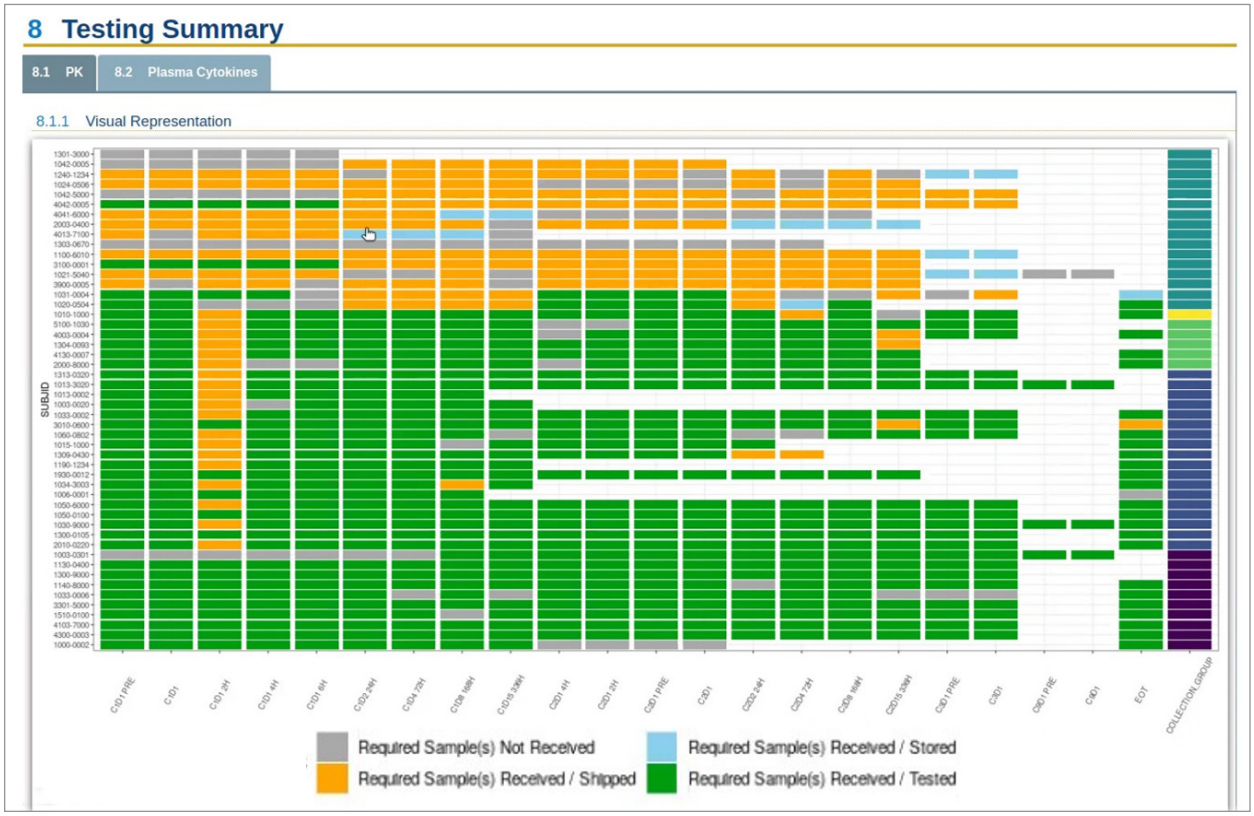
1.

Sample Status – Collection Through Testing

The report sample below provides a comprehensive status summary of all samples being tested in secondary labs (see Figure 4). Teams need to know at a glance which samples are completely missing from secondary labs (gray), which samples are in transit (orange), which samples are in storage (blue), and which have gone through testing by the lab (green). Using a report like the sample below to maintain continuous visibility allows teams to address discrepancies before more serious issues occur and optimize use of secondary lab resources.

> *"Our programs involve ultra-rare diseases, so every sample is important. We have to ensure that the right sample got to the right lab. But collections happen at many different sites—and we have to do reconciliation manually, one sample at a time. It takes MONTHS."*
>
> —Clinical Operations Director
> Global Biopharmaceutical Company

Figure 4 | **Example of Sample Status Report**

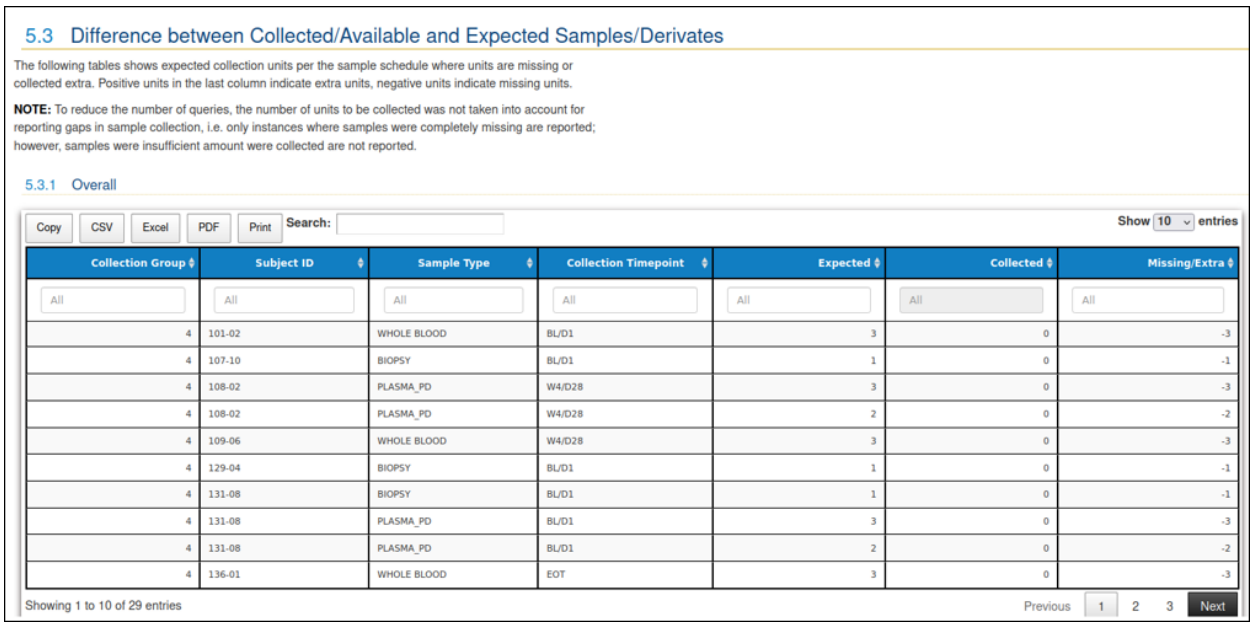


2.

Missing Samples

It is critical to CRO and sponsor teams to have the ability to identify missing samples. To identify missing samples, clinical operations teams first extract information protocols, protocol revisions, informed consent forms (ICFs), and optional consent forms to gain visibility into what samples and derivatives are expected to be collected. These expectations are then compared with actual collection data to generate a missing samples report (see Figure 5), which allows clinical operations teams to intervene during the trial to avoid losing critical biospecimens and data. This has historically been a complex and sometimes manual process. The sample report below, from the QuartzBio® platform, solves this problem effectively.

Figure 5 | **Example of Missing Samples Report**



5.3 Difference between Collected/Available and Expected Samples/Derivates

The following tables shows expected collection units per the sample schedule where units are missing or collected extra. Positive units in the last column indicate extra units, negative units indicate missing units.

NOTE: To reduce the number of queries, the number of units to be collected was not taken into account for reporting gaps in sample collection, i.e. only instances where samples were completely missing are reported; however, samples were insufficient amount were collected are not reported.

5.3.1 Overall

Copy CSV Excel PDF Print Search: Show 10 entries

Collection Group	Subject ID	Sample Type	Collection Timepoint	Expected	Collected	Missing/Extra
4	101-02	WHOLE BLOOD	BL/D1	3	0	-3
4	107-10	BIOPSY	BL/D1	1	0	-1
4	108-02	PLASMA_PD	W4/D28	3	0	-3
4	108-02	PLASMA_PD	W4/D28	2	0	-2
4	109-06	WHOLE BLOOD	W4/D28	3	0	-3
4	129-04	BIOPSY	BL/D1	1	0	-1
4	131-08	BIOPSY	BL/D1	1	0	-1
4	131-08	PLASMA_PD	BL/D1	3	0	-3
4	131-08	PLASMA_PD	BL/D1	2	0	-2
4	136-01	WHOLE BLOOD	EOT	3	0	-3

Showing 1 to 10 of 29 entries Previous 1 2 3 Next

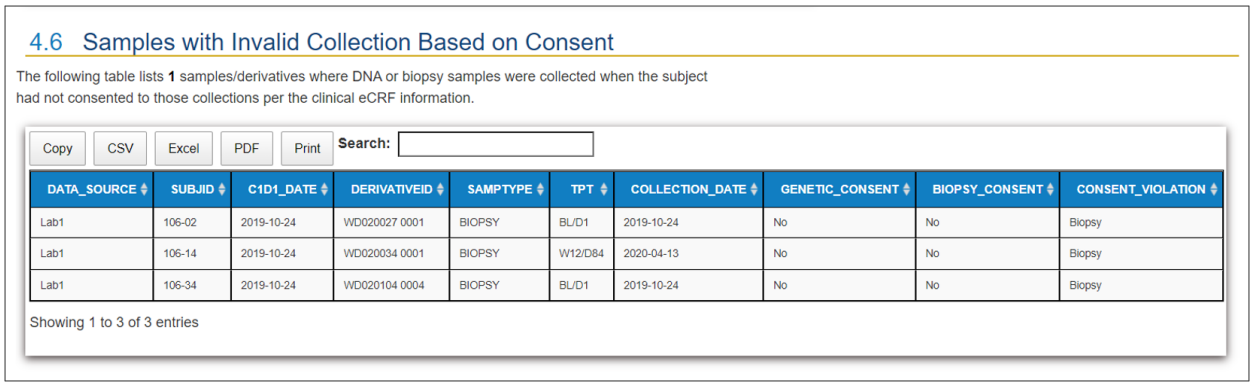
3.

Samples with Potential Consent Violations

When managing complex sample inventories across labs and repositories, clinical operations teams often spend time manually cross-referencing inventory data with informed consent forms and protocols to detect and report instances where a sample was collected but consent was not obtained.

In the example report below (see Figure 6), the study had two optional consents, one for genetic testing and another for biopsy sampling. The report shows that three subjects did not consent to biopsy, but a biopsy was taken, violating consent.

Figure 6 | **Example of Sample with Consent Violation Report**



4.6 Samples with Invalid Collection Based on Consent

The following table lists **1** samples/derivatives where DNA or biopsy samples were collected when the subject had not consented to those collections per the clinical eCRF information.

Copy CSV Excel PDF Print Search:

DATA_SOURCE	SUBJID	C1D1_DATE	DERIVATIVEID	SAMPTYPE	TPT	COLLECTION_DATE	GENETIC_CONSENT	BIOPSY_CONSENT	CONSENT_VIOLATION
Lab1	106-02	2019-10-24	WD020027 0001	BIOPSY	BL/D1	2019-10-24	No	No	Biopsy
Lab1	106-14	2019-10-24	WD020034 0001	BIOPSY	W12/D84	2020-04-13	No	No	Biopsy
Lab1	106-34	2019-10-24	WD020104 0004	BIOPSY	BL/D1	2019-10-24	No	No	Biopsy

Showing 1 to 3 of 3 entries

The three reports shown above are among the most critical reports needed by clinical and biospecimens operations teams in rare disease therapeutic development programs to:

1. Project forward-looking sample collections and assay results data availability
2. Confirm appropriate sample status prior to data generation
3. Monitor sample or consent expiry

Operating from a Single Source of Truth: Virtual Sample Inventory Management Across the Specimen Lifecycle

Access to continuously updated data and a collaborative platform would enable teams to work from a unified data set. With the QuartzBio® virtual Sample Inventory Management (vSIM) solution, all team members across the organization, across functions, can tap a centralized, reliable source of sample information. QuartzBio's data ingestion and harmonization pipelines continuously integrate sample information from multiple sources as it is generated, automatically perform edit checks, and highlight data inconsistencies.

In addition, QuartzBio's solution allows sponsors to maintain robust visibility across all the clinical samples generated in their portfolio. They can digitally monitor samples across their entire lifecycle, from collection and processing through testing and long-term storage.

Access and insight are critical to project teams and program leads. Real-time access to a single source of truth for samples in inventory empowers teams to make more informed decisions about their project/program's next steps.

Clinical operations teams can generate sample status, missing samples, and samples with potential consent violations reports on demand, providing up-to-date information on potential data issues as they arise throughout the course of a study, increasing data quality and maximizing the value of biospecimens.

Rare Disease Data and Sample Management Platform

Maintaining robust visibility into study data and biospecimens is critical for identifying potential issues or discrepancies and intervening in a timely manner. Precision for Medicine's proprietary QuartzBio® enterprise data platform is designed to integrate and organize diverse data sets and deliver real-time insights to maximize data generation potential.

RESEARCHING THE DEPTHS OF RARE DISEASES

Rare and ultra-rare indications face unique challenges, from the complexities of patient identification to those of advanced treatments, like cell and gene therapies. Identifying the right partner is essential to success in this nuanced space. Because no two studies, indications, or patients are the same, Rarefied Thinking presents sponsors with an opportunity to deliver true precision medicine that can change the lives of individuals and their families.

With this diverse, cross-functional perspective, Precision's experts are equipped to help you solve the most complex challenges at every stage of development, from research to realization to real-world impact.

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