METHODOLOGY PAPER

HEALTHCARE RESEARCH · RARE DISEASE & PATIENT RECRUITMENT

Rare-Disease Patient & *Caregiver* Sample

A Methodology for Ultra-Low-Incidence Recruitment

A method designed for volume cannot reach an audience defined by scarcity. This is a vendor-neutral guide to recruiting rare-disease patients and caregivers with rigour and care — a multi-source recruitment funnel under one screener, confirmed-diagnosis validation, and the respondent dignity and ethics that sensitive ultra-low-incidence research demands.

PUBLISHED BY
CatalystMR Research Team

SERIES
Methodology Papers

READING TIME
~17 minutes

EDITION
2026

CATALYSTMR
transforming market research data into marketing solutions

ABSTRACT

Rare-disease patient research demands a different recruitment strategy than standard consumer or healthcare studies. Low prevalence, diagnosis complexity, caregiver involvement, and privacy sensitivity mean an audience defined by scarcity and lived experience — one a volume-driven method will fail.

This paper concentrates on the discipline that decides a rare-disease study: **recruitment**. It builds a multi-source **recruitment funnel** that reaches widely but admits narrowly through one screener; sets the funnel's central gate as **confirmed diagnosis** rather than self-reported symptoms; treats **respondent dignity** as part of the method, not a courtesy; and sets out the **ethics and consent** that sensitive patient and caregiver research requires. Feasibility, mode choice, and post-field QC are covered in companion papers; here they appear only where recruitment touches them. Throughout, the goal is the same: not maximum volume, but verified relevance.

CONTENTS

WHO THIS IS FOR

Client-side healthcare and patient-insights teams and pharma/biotech researchers commissioning rare-disease studies; **agency researchers** designing and fielding them; and **procurement and strategy leaders** evaluating partners for ultra-low-incidence patient work. No prior fieldwork expertise is assumed; practitioners can skip to Section 02.

01 THE PROBLEM

Rare-disease audiences are too small for broad-panel targeting alone.

A method built for volume cannot reach an audience defined by scarcity. Rare-disease patients and caregivers are, by definition, a tiny share of any panel, and qualifying them is not a matter of casting a wider net — it is a matter of reaching the right people and confirming, carefully, that their experience is real. Four characteristics make these studies unlike any standard consumer or healthcare project.

DRIVER 1

Low prevalence

The condition is rare enough that broad-panel targeting alone cannot produce the sample.

DRIVER 2

Diagnosis complexity

Confirming a genuine, accurate diagnosis is harder than asking a yes/no symptom question.

DRIVER 3

Caregiver involvement

The respondent may be a patient, a caregiver, or both — each a distinct, valid voice.

DRIVER 4

Privacy sensitivity

The subject matter is personal; participation carries real emotional weight.

Medical qualification is necessary, but not sufficient

Even when a respondent qualifies medically, they may still not match the study's requirements — around therapy experience, payer type, disease severity, or specialist care. A confirmed diagnosis is the entry condition, not the whole specification. The study target must resolve these additional dimensions explicitly, or a medically-genuine respondent can still be the wrong respondent for the research question.

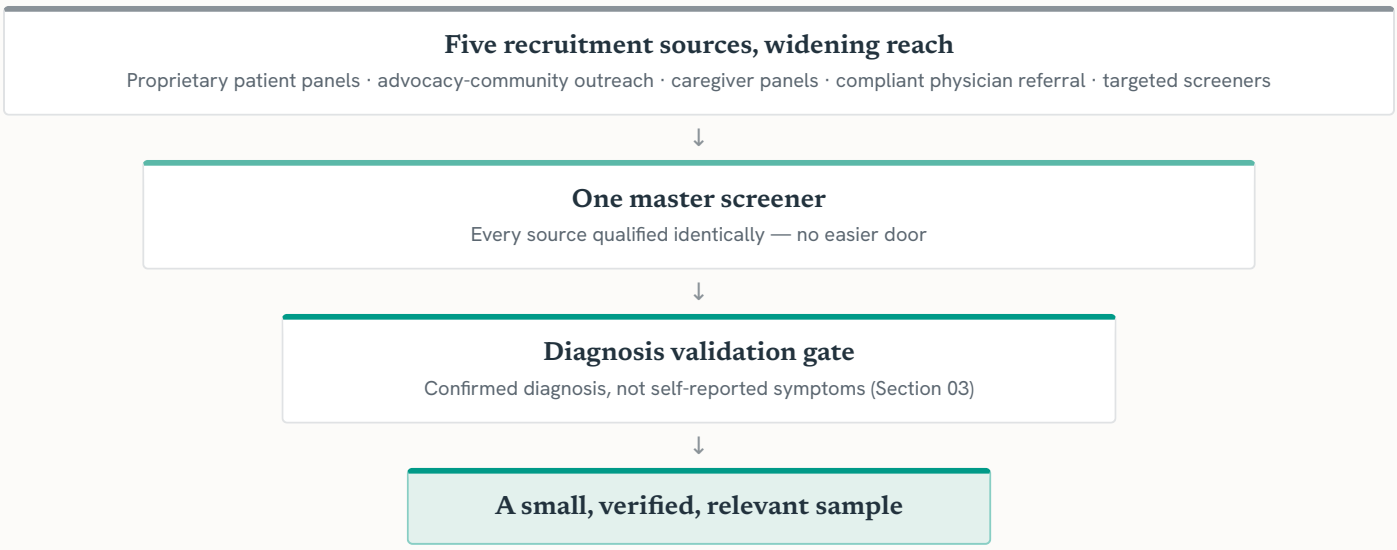
THE THROUGHLINE

The goal is not maximum volume; it is verified relevance. Everything that follows — the recruitment funnel, the diagnosis gate, a dignified respondent experience, and the ethics of sensitive research — serves that single principle: the right lived experience, confirmed, and treated with care.

02 RECRUITMENT

Reach widely; admit narrowly.

Successful rare-disease studies rarely come from a single panel. They combine several sources — each reaching a part of the audience the others cannot — and then pass everyone through one screener and one standard of proof. The funnel below is the shape of that discipline: many doors in, a single qualifying gate, a small verified sample out.



Why the gate, not the doors, defines quality

Multiple sources widen the top of the funnel, but the screener must keep the bottom narrow. A study that loosens its qualification to fill quota from an easy source has not solved its feasibility problem — it has hidden it inside the data. Let the sources compete for reach while one screener decides, identically, who qualifies.

Each source has its own duty of care

Advocacy and physician-referral routes reach engaged communities, but they carry the trust of those communities and clinicians with them; any compliant referral path must be handled accordingly. Feasibility for the whole funnel — how rare the cell is, and how long it will take — is modelled in **Paper No. 134**; mode choice for hard-to-reach cases in **No. 135**.

PRINCIPLE

Judge a recruitment plan by the relevance of who it admits, not the volume it can reach. A small, verified, well-matched sample is worth far more than a larger one diluted to hit a number.

03 THE GATE

Confirm the diagnosis — don't accept the symptom.

The funnel's central gate is diagnosis validation: separating a confirmed diagnosis from self-reported symptoms. Symptoms can be guessed, misattributed, or claimed; a genuine diagnosis, treatment history, and specialist relationship are far harder to fabricate — and far more useful to confirm. Validation should test the claim from several angles and look for consistency across them.

VALIDATE AGAINST

Confirmed diagnosis

A specific, accurate diagnosis; a coherent treatment history; the right specialist type; genuine medication familiarity. Evidence that holds together across the screener and the main survey.

DO NOT ACCEPT ALONE

Self-reported symptoms

A symptom checklist a respondent can pattern-match to, with no corroborating treatment, specialist, or medication detail. Necessary context, but never sufficient proof on its own.

What thorough validation looks at

- **Diagnosis confirmation questions** — specific, not a symptom checklist.
- **Treatment history** — coherent with the stated condition and stage.
- **Specialist type** — the kind of clinician this condition is actually managed by.
- **Medication familiarity** — knowledge consistent with genuine experience.
- **Consistency checks** — answers that must agree across screener and main survey.
- **Open-ended responses** — lived experience that is hard to fabricate.

WHY OPEN-ENDS MATTER MOST HERE

Open-ended responses help distinguish real lived experience from respondent guessing or fraudulent qualification. In an audience this small, a handful of carefully-read verbatims about the day-to-day reality of a condition can reveal more than any closed question — and protect the study from the rare but costly bad qualifier. (Post-field auditing of those verbatims is detailed in Paper No. 137.)

04 RESPONDENT CARE

Design for the person, not just the data point.

Rare-disease respondents may be patients, caregivers, or both — and they are often sharing something personal and difficult. A study that treats them as a data point will lose them; one that treats them with respect earns better data and honours the contribution. Respondent experience is not a courtesy in this work; it is part of the method.

CARE 1
Plain language
Clear, jargon-free wording that any patient or caregiver can follow with confidence.

CARE 2
Respectful tone
A tone that acknowledges the reality of the condition and the person describing it.

CARE 3
Mobile & flexible
Mobile-friendly design and flexible completion options that fit around real lives.

CARE 4
Fair incentives
Incentives that reflect the difficulty and emotional labour of taking part.

Patients and caregivers are different voices

A caregiver's account of a condition is not a substitute for a patient's, nor the reverse — each sees a different part of the journey. The FDA's Patient-Focused Drug Development guidance treats patient and caregiver experience as distinct, valuable inputs; a well-designed study decides deliberately whose perspective it needs, captures which role the respondent holds, and writes the instrument so both can answer truthfully without being forced into the other's frame.[1]

Sensitivity is a design requirement

When the topic is sensitive, the emotional labour of participation is real, and the instrument should reflect it: realistic length, the ability to pause or complete flexibly, and incentives that recognise what is being asked. Respect is not only ethical — it directly reduces abandonment in an audience you cannot easily replace.

PRINCIPLE

In an audience this scarce, every respondent who abandons is one you may not be able to replace. Designing for dignity is also the most practical way to protect feasibility.

05 ETHICS

Sensitive health research carries duties beyond data quality.

Rare-disease research collects some of the most sensitive personal data in market research — diagnosis, treatment, lived experience — often from people in difficult circumstances, and sometimes from caregivers answering on another's behalf. The ICC/ESOMAR Code's duty of care to participants, and the patient-centred spirit of the FDA's Patient-Focused Drug Development guidance, both point the same way: the ethics are part of the method, not an afterthought.[1,2]

What sensitive-audience ethics require

- **Informed, specific consent** — clear about what is collected, why, and how it will be used, before participation.
- **Data minimisation & protection** — collect only what the study needs; store sensitive health data securely and siloed from response data.
- **Caregiver & proxy clarity** — record who is answering, and respect the boundaries of answering for another.
- **A genuine right to withdraw** — without penalty, and with incentives handled fairly if a respondent stops.

Vulnerability and adverse events

Some respondents are seriously ill or recently diagnosed; the instrument and any interviewer contact should be designed with that in mind. Where a study touches a specific therapy, researchers should also know their obligations around any **adverse event** a respondent mentions — the requirement to recognise and route it appropriately is a standing feature of healthcare-research conduct, and should be agreed with the client before fielding.

ETHICS PROTECTS THE DATA, TOO

Treating participants ethically is not in tension with data quality — it underwrites it. Respondents who feel safe, informed, and respected give fuller, more honest answers and are likelier to complete; in an audience you cannot easily replace, that is also the most practical route to a usable sample.

06 THE CHARTER

What every rare-disease respondent is owed.

The method in this paper reduces to a short charter — six commitments a buyer should expect of any rare-disease study, and hold a provider to. They are the recruitment funnel, the validation gate, the respondent experience, and the ethics of the preceding sections, expressed as obligations to the person taking part.

One honest qualifying gate

Reached through whichever source, every respondent is qualified by the same screener — never admitted through an easier door to fill quota.

Confirmed, not assumed

Eligibility rests on a confirmed diagnosis and corroborating detail, so a respondent's genuine experience is recognised and a guess is not.

Their own voice

Patients and caregivers are captured as distinct roles, and neither is forced into the other's frame.

An instrument designed with care

Plain language, realistic length, flexible completion, and incentives that reflect the difficulty and emotional labour of taking part.

Consent and control over their data

Informed, specific consent; data minimised, secured, and siloed; and a genuine right to withdraw without penalty.

Verified relevance over volume

The study commits to a small, well-matched, defensible sample rather than a larger one diluted to hit a number.

WHERE THIS SERIES GOES DEEPER

No. 134	**Feasibility & incidence.** Modelling how rare the cell is, and how long recruitment will take, before launch.
No. 135	**Mode choice.** Reaching cases who won't self-complete online via telephone (CATI) or mixed-mode.
No. 137	**Quality control.** The eleven-point framework, including the open-end auditing that protects a small sample.

▲ CONCLUSION

Verified relevance, treated with care.

Rare-disease recruitment rewards rigour and respect in equal measure. Reach widely across multiple sources but admit narrowly through one screener; make confirmed diagnosis the gate; design a respondent experience worthy of the people taking part; and hold to the ethics that sensitive health research demands. Each commitment protects the others, and together they convert an ultra-low-incidence audience into a small, verified, well-matched sample you can stand behind. The FDA Patient-Focused Drug Development guidance, the ICC/ESOMAR Code, and the ISO 20252 framework exist precisely so buyers can ask for this rigour in consistent terms — and recognise it when they see it.[1,2,4]

§ REFERENCES

[1] U.S. Food & Drug Administration. **Patient-Focused Drug Development Guidance Series** (four methodological guidances on collecting patient and caregiver experience data). FDA, 2018–2025. fda.gov

[2] ICC/ESOMAR. **International Code on Market, Opinion and Social Research and Data Analytics.** ICC & ESOMAR. (Duty of care to participants; informed consent; data protection.)

[3] ESOMAR. **37 Questions to Help Buyers of Online Samples.** Amsterdam: ESOMAR, 2023. esomar.org

[4] International Organization for Standardization. **ISO 20252:2019 — Market, opinion and social research, including insights and data analytics — Vocabulary and service requirements.** Geneva: ISO, 2019.

Sources are cited for the patient-centred methodology, ethics, and quality frameworks referenced; the FDA PFDD guidance is cited for its treatment of patient and caregiver experience data as distinct, valuable inputs, not for any figure. Methodological principles stated without a citation (e.g. that confirmed diagnosis is stronger than self-reported symptoms, or that scarcity raises the cost of each lost respondent) are definitional to patient-sample practice. This paper publishes no prevalence, incidence, fraud, or cost figures; any such metric should be modelled from the specific study's own condition, criteria, and confirmed cases. Photography: National Cancer Institute (Unsplash License) and Wikimedia Commons.

§ ABOUT

CatalystMR

CatalystMR is a global market-research panel and fieldwork partner specialising in **hard-to-reach patient, healthcare, and niche audiences**. For rare-disease work we combine proprietary patient and caregiver sample with advocacy outreach, compliant physician referral, and live telephone (CATI) capability — under one screener, one QC standard, and one point of contact.

We publish our own responses to ESOMAR's 37 Questions and design diagnosis validation, respondent care, and participant ethics into every patient engagement rather than treating quality as a post-field cleanup.

Compliance posture: our methodology is aligned to the ESOMAR Code and Guidelines and the ISO 20252 framework, and we are certified under the EU–U.S., UK, and Swiss Data Privacy Frameworks, with personal data siloed from response data.

Tell us your condition, criteria, and target completes, and we'll return a recruitment plan and a modelled feasibility range built from confirmed cases — typically within 24 hours — not a hopeful number.

RARE DISEASE · PATIENT RECRUITMENT · CAREGIVERS · ETHICS & CONSENT · FDA PFDD · ISO 20252

sales@catalystmr.com
1-800-819-3130
catalystmr.com