

A Systematic Approach to Early Patient Engagement in Clinical Trials

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Rare disease clinical trials pose **unique challenges.**

- Unique patient needs
- Small, widely dispersed patient populations
- Few diagnostic tools
- Complex regulatory pathways



400 million
people
worldwide
living with a
rare disease.¹



**Less than
10% of rare
diseases**
lack an
approved
treatment.²

1. Global Genes. Rare Disease Facts. Accessed 23 Feb 2026. <https://globalgenes.org/rare-disease-facts/>

2. Fermaglich LJ, Miller KL. A comprehensive study of the rare diseases and conditions targeted by orphan drug designations and approvals over the forty years of the Orphan Drug Act. Orphanet J Rare Dis. 2023;18(1):163. Published 2023 Jun 23. doi:10.1186/s13023-023-02790-7

Adapting the Tufts' Patient Friction Coefficient model to rare disease trials

Collecting **direct feedback** from the patient community and continuously applying those learnings into our rare disease clinical trial design.

These structured patient perspective exchanges touch on:

Trial Awareness

Trial Enrollment

Trial Participation

Trial Follow-Up



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Assessing Participation Burden in Clinical Trials: Introducing the Patient Friction Coefficient



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Using the Patient Friction Coefficient (PFC) Framework to embed patient insights directly into the protocol.

Internal Alignment

Internal stakeholders

Session participants

Engagement planning

Patient Perspective Exchange Sessions

Disease journey

Proposed trial design presentation

Q/A & Feedback sharing

Insights Readout

Review feedback

Incorporate insights into final protocol

Develop recruitment strategy based on input

Insights to Actions

What we heard

What we changed

**Trial
Awareness**

**Trial
Enrollment**

**Trial
Participation**

Others

Value and Impact

**Patient-centered
trial protocols**

**Recruitment and
retention enablers**

**Operational
readiness**

**Time and cost
savings**

**Quality and
consistency**

Cultural shift



Thank you!

Questions?

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