

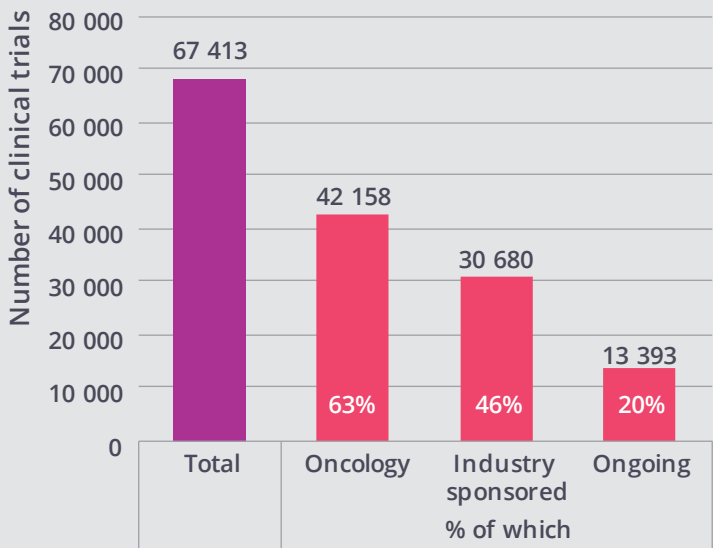
Rare Disease Trial Strategies

Opportunities and Challenges

20%

Rare Diseases Carry a Large Collective Footprint

While rare diseases are individually uncommon, the combined burden is vast. One in five of all known rare disease studies are currently ongoing, as trial activity has steadily increased through the last decade.

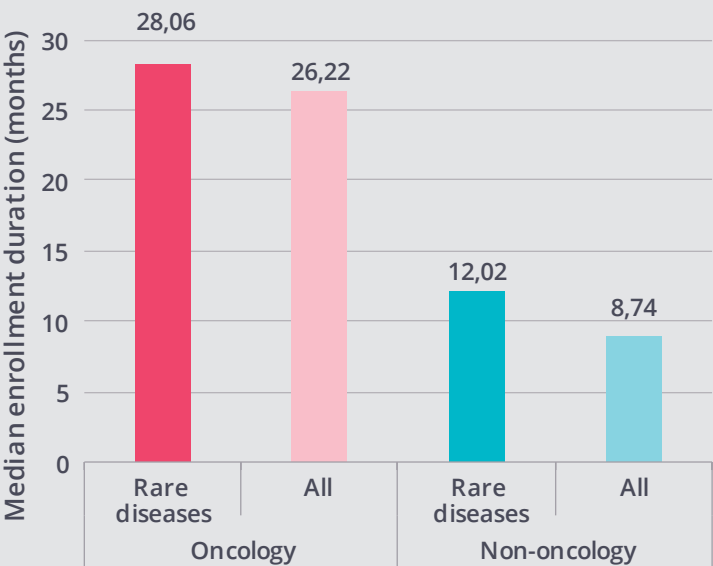


Source: Trialtrove, October 2021

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Studies for Rare Diseases Typically Take Longer to Recruit and Conduct

Because the enrollment duration of rare disease trials is typically longer, this adds to the cost burden of rare disease R&D.



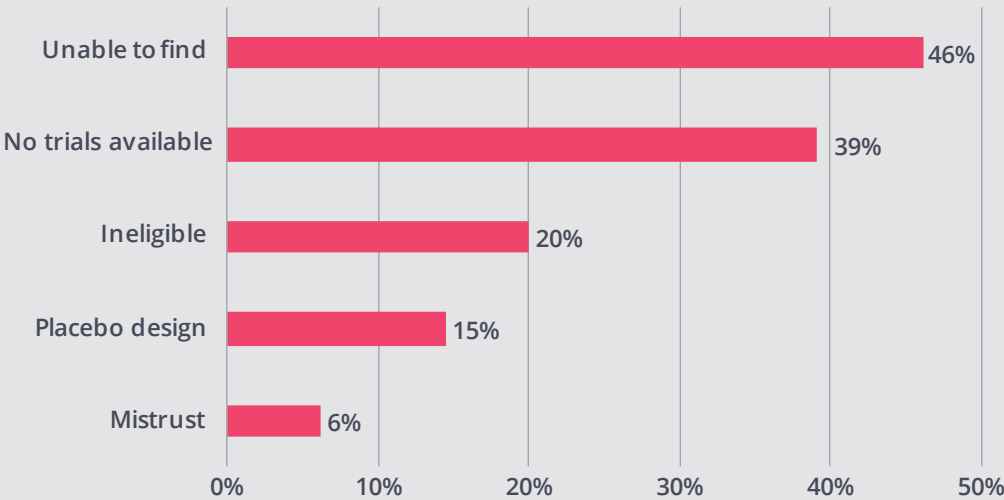
Source: Trialtrove, October 2021

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Needle in a Haystack

The most prevalent obstacles to participation are undoubtedly being unable to locate relevant trials that are an appropriate match to both geographical location and/or condition/disease.

Why Have You Not Participated in a Trial?



Source: Informa Pharma Intelligence-Rare Patient Voice Survey 2021

Strategic Recommendations for Sponsors

1

Gather Insights From Experts for Patient-Centric Studies

Physicians are a popular source among patients for trial awareness and information. Along with clinical experts, they can play a pivotal role in the trial design process, from protocol development and study feasibility, to protocol development to clinical endpoints. Physicians are also a gateway into patient insights and unmet needs.

2

Build Trial Awareness From the Outset

Successful trial awareness should have a multi-pronged approach that targets the important stakeholders: physicians, investigators, and patients themselves.

3

Embrace New End-To-End Recruitment Models

A centralized approach to awareness and enrollment can track patient interest, eligibility, and referrals. This hub can also double as a communication portal, supporting an overall positive trial experience before, during and after a clinical trial.

Citeline Connect

Everything you need to connect the dots between patients and clinical trials

Citeline Connect is the all-in-one clinical trial recruitment platform that accelerates trial enrollment via innovative technology and services.



Trial Website



Enrollment Hub



Communication Portal

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