CLINICAL TRIALS ACCESS

Overcoming the Earliest Barrier to Treatment

GAfPA
Global Alliance for Patient Access

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Scientific discovery is the first step toward new treatments and breakthrough cures.

Introduction

Patient access starts with medical research. Clinical trials are an essential part of developing the new medicines, devices and diagnostics that improve health and quality of life worldwide.

Clinical trials determine whether new treatments are safe and effective. Robust enrolment is necessary for that research to succeed, but several challenges stand in the way, including:

- The public's lack of clinical trials awareness
- Costs incurred by participating patients
- Lack of engagement by health care providers
- Persistent health disparities

These impediments are the first and most fundamental barrier to patients accessing the next generation of innovative treatments and cures.

While the barriers are multifaceted, so too are the opportunities for policymakers, health care providers and patient advocates to improve access and advance medical innovation.[1]

Clinical Trials at a Glance

In many respects, clinical trials are a global success story.

Roughly 419,000 studies are underway right now, with locations in all 50 U.S. states and 221 countries worldwide.[2] Western Europe has the highest rate of new trial launches, followed by Eastern Europe and the Asia-Pacific region.[3] More than $160 billion is expended on research and development for clinical trials each year, resulting in 50 new therapies approved by the United States Food and Drug Administration in 2021 alone.

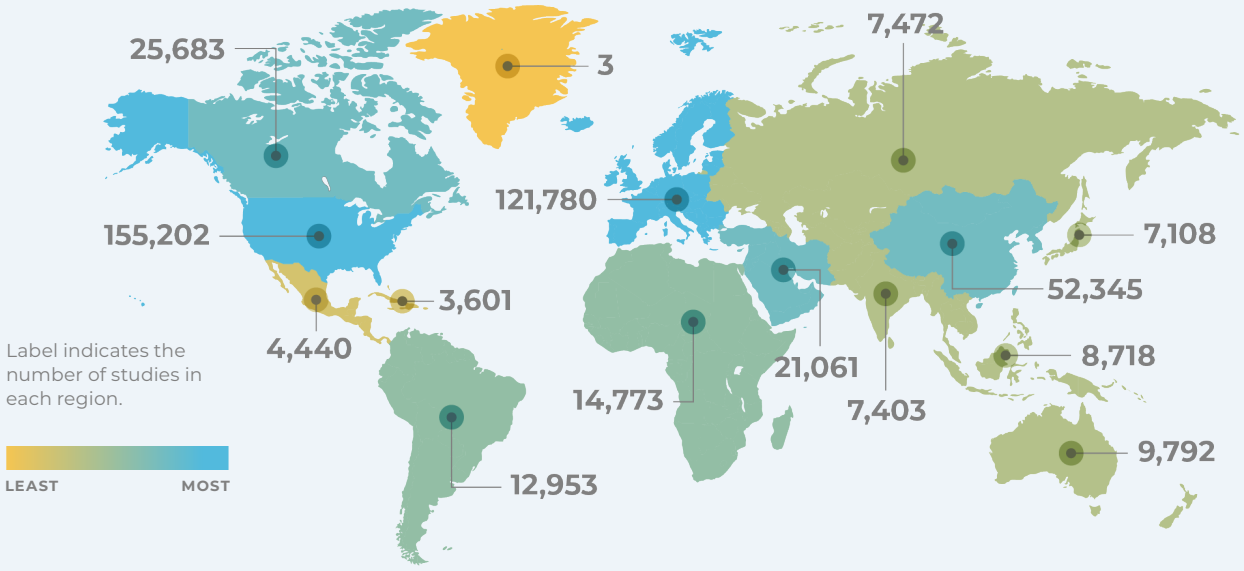
These impressive sums reflect the time and commitment needed to conduct clinical research. A trial for a new medicine can require tens to hundreds of millions of dollars in investment, hundreds or thousands of eligible study participants, and years or even decades of research to prove safety and efficacy. There are also indirect costs. These include expenditures on research and lines of scientific inquiry that prove unsuccessful, as well as unreimbursed expenses incurred by study participants, their families and caregivers.

These investments — and researchers' persistence — have delivered new promise for patients. In the last decade alone, examples of breakthrough cures and treatments include immuno-therapies to cure childhood cancers, cystic fibrosis treatments that dramatically extend life expectancy and life-changing medicines for often debilitating conditions like migraine. Millions of lives were saved when the decades of collective research in developing mRNA technology yielded a vaccine against COVID-19 in record time.

Clinical trials are underway in 221 countries worldwide.

Rare disease research offers a particularly compelling success story. While the likelihood of approval for new drugs across the board was about 8% from 2011 to 2020, treatments for rare diseases demonstrated remarkable success, seeing a likelihood of approval of 17%.[4]

By The Numbers: **Global Clinical Trials**[5]



Enrolment Challenges

For every success story, however, scores of clinical trials are cut short due to low enrolment.

Studies have found that 12% of listed trials are terminated—meaning they halt enrolment prematurely. The most common reason is low recruitment.[6] Meanwhile, nearly 80% of clinical trials fail to meet enrolment timelines, delaying the development of potentially life-saving treatments.[7]

Granted, drug development is a long process. It can require 10 years or more to take a potential treatment from "bench to bedside." Estimates of total cost vary but can be as high as $2 billion when factoring in post-approval studies and opportunity costs. Slow recruitment derails deadlines, raises costs, and consumes more time and resources than any other aspect of clinical trials. Recruiting participants takes up to 30% of development timelines, with around $1.2 billion in investment. Recruitment difficulties lead to delays of anywhere from one to six months.[8]

For each day a trial is delayed, losses mount. With 11% of clinical research sites failing to enrol even a single participant, and 37% of sites under-enrolling, recruitment strategies are a pivotal component of successful trials.[9]

One in 10 trials are terminated, most often due to low enrolment.

Understanding Access Barriers

Access challenges, including enrolment struggles, stem from several factors.

Lack of Awareness

Many patients lack general awareness about clinical trials. Data show that higher education levels, higher yearly incomes and internet use are associated with increased awareness.[10] Yet these advantages are not available to all. Perhaps that's why 40% of surveyed adults admit not fully understanding clinical trials.[11] This knowledge gap can undercut patients' opportunity to participate and can worsen economic and health disparities.

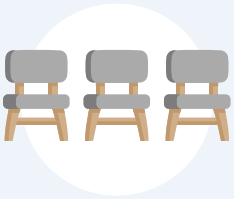
Insufficient Engagement by Health Care Providers

Engagement by health care providers is another concern. Studies show that most physicians are comfortable discussing clinical trials with their patients, but fewer than 1% actively refer patients to studies. Keeping current on trial opportunities is a key factor. Physicians report lacking access to current trial information, and about one-third say they simply don't have time to learn about active trials.[12]

Lack of Diversity

Recruiting a diverse group of patients for trials is also difficult — but essential. Patients may experience the same disease in very different ways. A diverse sample gives researchers a better chance of measuring the efficacy and identifying side effects specific to patient subpopulations.

By The Numbers: **Clinical Trials Awareness**

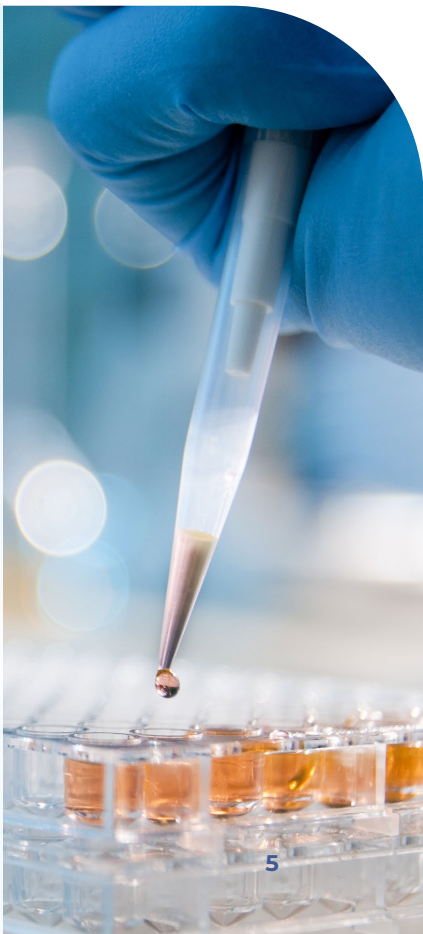


11%
of sites fail to enrol **even a single patient**

40%
of surveyed adults **don't understand clinical trials**

37%
of sites **do not meet** their enrolment goals

32%
of surveyed adults say **they'd consider participating**— after they understand what a clinical trial is[13,14]



People of colour are **under-represented in more than half** of phase III trials in the European Union.

But enrolling participants from diverse and underserved communities has historically been challenging or simply not prioritised by researchers. People of colour are under-represented in more than half of phase III trials in the European Union.[15] Diversity is also lacking in site personnel. In Europe, fewer than 10% of clinical trials personnel are from minority populations. Research shows that the two factors are interconnected. Trials that have a diverse staff are more likely to view participant diversity as a success factor and to prioritise recruiting diverse participants.[16]

Costs & Time Commitment for Participants

The time commitment, financial costs and emotional toll incurred by patients seeking to enrol in a study often pose yet another barrier to clinical trials access. These burdens include the challenge of locating a trial, the travel and distance required to reach a study site, the time commitment of navigating the extensive paperwork and screening, and the unreimbursed costs the patient might incur.

Too often, patients are simply overwhelmed by the bureaucratic process of identifying and enrolling in a trial. Informed consent, for example, is a lengthy process during which trials can lose willing participants because they need urgent treatment.

While trial sponsors generally cover all research-related costs and special testing, participants may still face indirect costs. That may include lost income due to taking time off work, child-care and unreimbursed travel expenses, and out-of-pocket medical costs.

Patient Concerns & Uncertainty

Fear and anxiety can impact participation too. Many patients are already reeling from the distress, symptoms and implications of their disease. Considering whether to participate in a clinical study introduces additional worries, such as a fear of taking an untested medicine or receiving a placebo, potentially missing out on a proven medication during a crucial time.

Improving Clinical Trials Access

Improving clinical trials access requires a concerted effort by all stakeholders.

- **Investors.** About 90% of investment comes from life science companies, but medical research requires greater public-sector commitment.
- **Patients.** Trial design is increasingly patient-centred, but the best designed study cannot be successful without enroled patients.
- **Health care providers.** Tens of thousands of providers engage in medical research, but the vast majority could do more to enhance patient access to clinical trials.
- **Policymakers.** Policymakers regulate clinical trials but should treat clinical trial access as a public health priority.

Policymakers in particular can enhance public awareness and improve patient access to trials in several ways.

Support Patient Enrolment

Policymakers can make it easier for patients to navigate clinical trials. Patient-friendly online databases are critical. Online databases of open trials should be as easy to navigate as an e-commerce website.

Policymakers can also provide funding for clinical trial navigators, whose services can ease enrolment and address patients' questions as they arise. General enrolment rates for patients who receive patient navigation is as high as 95%. In studies on how patient navigation impacts minorities' enrolment in clinical trials, strategies such as streamlining enrolment and reimbursing patient expenses have been shown to achieve up to 86% enrolment of African Americans who had been considering participation.[17]

Educate and Empower Providers

Policymakers should also empower health care providers to support patient enrolment.

That could mean incentivising providers to support patients in searching for and enrolling in studies, and to remain engaged as a part of the research care team. Improvements in electronic medical records are also critical. Records systems should be enhanced so that when providers log a diagnosis, they are linked directly to a database of clinical trials in which the patient may eligible to enrol.

Prioritise Diversity

Clinical trials often rely disproportionately on white male participants. This shortcoming has created gaps in researchers' understanding of diseases and conditions, preventive factors, and treatment effectiveness across populations.[18]

Policymakers should provide financial support, through grants and tax credits, for trials whose study design and enrolment targets promote diverse racial and ethnic participation and involve investigators of colour. Governments should also dedicate resources to outreach in communities of colour and to addressing economic disparities that preclude or discourage people of colour from participating in trials.

Make Participation a Public Health Priority

Medical research is an essential aspect of public health. To address any public health challenge, policymakers must first raise public awareness. That involves investing in campaigns to increase general awareness about the value of medical research and the opportunities for patients to enrol in studies. From anti-smoking to organ donation, awareness campaigns have longed played a role in policymakers' efforts to change public attitudes and behaviors.

Foster Increased Investment in Research

To create more opportunities for medical advances and breakthroughs, clinical research needs to continue to attract private-sector funding.

That requires public-private partnerships that incentivise investment, as well as policy solutions that reward innovation, strengthen protections for researchers' intellectual property, and promote study enrolment and access to new therapies.

Conclusion

Improving access to clinical trials will spur investment and reduce the costs of research that ultimately improves and extends the lives of patients across the globe.

Just as barriers to clinical trials access come in multiple forms, so do the policy solutions that will help patients overcome those barriers. Making clinical trials enrolment more efficient, encouraging diversity among participants, increasing health care providers' engagement and raising awareness amongst patients will improve access.

By working together to reduce barriers, patients, providers, researchers and policymakers can clear the way for robust clinical trials that yields the next generation of new drugs and life-saving treatments.

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