

# How are medicines developed?



Alzheimer's Disease International supports Alzheimer's disease clinical trials. We know that research is the engine that powers medical progress. Clinical trials provide opportunities for you to advance medical research and to contribute to improved treatments for future generations.<sup>1</sup>

Before a new medicine can be used for treating Alzheimer's disease, it is studied in clinical trials to determine if it is safe and effective in people.<sup>2</sup> There are many different types of clinical trials, some of which examine experimental medicines and some that do not include medicine at all.

- Treatment trials test medicines that are already being used, experimental medicines, or new combinations of treatments<sup>3</sup>
- Diagnostic trials find better tests or procedures for diagnosis<sup>3</sup>
- Screening trials test the best way to detect certain diseases<sup>3</sup>
- Behavioral trials focus on a patient's behaviors and relieving caregiver burden<sup>4</sup>

# How medicines are developed

A goal of clinical trials is to develop new medicines.<sup>2</sup> Even a clinical trial that does not produce positive results with a new medicine is still valuable,<sup>5</sup> as all results provide researchers with information to help develop new treatments for Alzheimer's disease.

Before any medication is used to treat a disease, it must be carefully tested in clinical trials to make sure that it is safe and that it works in people.<sup>2</sup>

Discovering a new medicine starts with an unmet medical need, such as the need for more effective treatments. After determining the unmet need, researchers identify specific targets for which experimental medicines can potentially work.<sup>6</sup>

There are many different approaches currently being researched for Alzheimer's disease; one of the most widely explored is beta amyloid.<sup>7</sup>

Each of these trials plays an important role in advancing our understanding of the disease, its diagnosis and, ultimately, its treatment.



## How new medicines are developed

#### The importance of clinical trials

The development of a new medicine starts in the laboratory, where researchers identify thousands of compounds that may play a role in Alzheimer's disease. Many years are spent testing and modifying these compounds to improve how they work and to decrease potential side effects. If this preclinical phase of research shows favorable results, clinical trials in people can begin.

Clinical trials are conducted in phases. At each phase researchers try to answer different questions about the experimental medicine they are researching. Progression from one phase to the next generally requires success in the prior phase.

#### **Preclinical**

An experimental medicine first goes through rigorous testing in the laboratory. If the experimental medicine has the potential to treat an illness, it can move on to clinical testing in people. Many experimental medicines tested in the laboratory never advance to clinical trials in people.<sup>8</sup>

#### Phase 1–3 trials

Testing in people is done in three phases of clinical trials.

The goal of **Phase 1** is to test safety.

**Phase 2** tests how well an experimental medicine will work to treat Alzheimer's disease.

In **Phase 3** the experimental medicine is tested in large numbers of people to confirm how well it works. Phase 3 trials may compare the experimental medicine to another commonly used medicine for Alzheimer's disease treatment, such as a cholinesterase inhibitor.<sup>1,2,8</sup>

#### Registration

Results from all phases of the clinical trials are provided to the health regulatory authorities to evaluate the risks and benefits of the experimental medicine and determine whether to approve the medicine for broad use. If the regulatory authorities approve the new medicine, it can then be prescribed by physicians to their patients.<sup>8</sup>

#### Phase 4 clinical trials

**Phase 4** trials are conducted after a treatment has already been approved. In Phase 4 trials, researchers collect additional valuable information, such as the long-term risks or other benefits of the medicine.<sup>2,8</sup>

# The clinical trial process

#### AVERAGE LENGTH OF PROCESS 13 YEARS<sup>6</sup>······



# The importance of clinical trials

Clinical trials would not be possible without volunteers. To make sure that trials are conducted ethically, there are many rules and standards for how a trial proceeds, including: <sup>9</sup>

- The use of highly qualified researchers
- External and independent review by an institutional review board or an ethics committee
- Ongoing monitoring of all trial sites
- The use of informed consent documents that outline the risks and potential benefits of participation
- Ability for all volunteers to withdraw from the trial at any time

Clinical trials move medicine forward, helping researchers make advances in Alzheimer's disease treatment.

Without clinical trials, there can be no new treatments or cures.

## For more information

Alzheimer's Disease International provides general information on Alzheimer's disease at www.alz.co.uk. Please contact your national Alzheimer's association or visit the following websites:

- List of national Alzheimer's associations: www.alz.co.uk/associations
- EU Clinical Trials Register: www.clinicaltrialsregister.eu
- Listing of trials being conducted in the United States and around the world: ClinicalTrials.gov
- The US-based Alzheimer's Association: www.alz.org

### References

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- 4 Bill and Melinda Gates Foundation. Clinical Trial Fact Sheet. http://www.gatesfoundation.org/grantseeker/Documents/Clinical\_Trials.pdf
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- 9 World Health Organization. Guidelines for good clinical practice (GCP) for trials on pharmaceutical products. http://apps.who.int/medicinedocs/pdf/whozip13e/whozip13e.pdf



This content has been reviewed by an Alzheimer's Disease International steering committee and developed with support from Pfizer Inc. Alzheimer's Disease International: The International Federation of Alzheimer's Disease and Related Disorders Societies, Inc. is incorporated in Illinois, USA, and is a 501(c)(3) not-for-profit organization



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