

COVID-19 VACCINE: Clinical Trials and Safety

Talking Points

All pharmaceutical products that are used in the United States, including vaccines for COVID-19, must undergo stringent regulatory review before they are approved by the United States Food and Drug Administration (FDA).

The most important requirements for any product are safety and efficacy (how well it works).

Companies such as Pfizer, Moderna and AstraZeneca that are involved in the development of COVID-19 vaccines have to follow specific guidelines and testing protocols to ensure that their products will be safe and effective when administered to human beings.

Pharmaceutical Development Process

Prior to any human testing, the first process for vaccine development occurs in a company laboratory. In this “preclinical” development process, scientists employed by the company will create the vaccine molecule.

During the preclinical development phase and once a successful product is developed, the company will expand and implement its manufacturing process to produce a product intended for human use.

If the preclinical evaluations are positive, the company will file with the FDA for permission to allow for the first in-human trials.

There are three phases on clinical trials for vaccines:

PHASE 1

During Phase 1, a small group of healthy individuals are given several incremental doses of the vaccine to evaluate safety of the product in healthy humans.

These individuals may be given different levels of doses or multiple doses to assess safety. If the safety risk is assessed to be acceptable, the vaccine will be allowed to move to Phase 2 studies.

PHASE 2

In Phase 2, the vaccine’s efficacy is tested by providing some individuals with the vaccine and others with a placebo. For the COVID-19 vaccine, the Phase 2 tests included testing the vaccine for efficacy (did the subjects develop antibodies to COVID-19) in a small group of individuals who are at a higher risk of acquiring infection.

However, the number of people involved in Phase 2 trials does not capture the entire demographic of those who will be ultimately immunized as a part of a national vaccination effort. These early studies are too small to draw conclusions about the safety and efficacy for cohorts of all population groups based on age, race or other factors.



COVID-19 VACCINE: Clinical Trials and Safety

PHASE 3

To significantly expand the pool of those being tested, following the successful completion of a Phase 2 trial, the company will seek approval to continue with Phase 3. This trial phase will involve the largest and most comprehensive assessment of safety and efficacy of the vaccine product to be tested over a broad group of people who represent the general population.

For example, in the 2020 Pfizer/BioNTech mRNA vaccine for the Phase 3 trial, 44,000 total participants were selected from a broad range of demographics. Of these, 50% received the placebo and the other 50% received the actual mRNA vaccine.

Two doses were administered over a 28-day interval. The results from the Phase 3 trial for the Pfizer/BioNTech vaccine showed around 95% efficacy.

NDA AND EUA

Following successful Phase 3 studies, the company will then submit the New Drug Application (NDA) to the FDA for approval of the vaccine candidate. In addition to the full approval, the FDA allows companies to apply for Emergency Use Authorization (EUA) approval, which is a mechanism for approval by the regulatory agency to use the product in a defined population.

In the case of COVID-19 vaccines, the EUA is specifically designated for use in frontline and healthcare workers, and for use in elderly individuals, especially in nursing homes, who are most vulnerable to getting the infection.

During the Emergency Use Authorization, the safety and efficacy of the vaccines are continuously monitored.