



COVID-19 VACCINE INFORMATION

The National Organization for Rare Disorders (NORD) prepared this resource to address questions and concerns we have heard from the rare disease community regarding COVID-19 vaccines, including information about vaccine safety, efficacy and availability. This information is based on our January 15 webinar with Food and Drug Administration (FDA) and Centers for Disease Control and Prevention (CDC) leaders, which [you can listen to in its entirety here](#).

Updated February 2, 2021

1. What Vaccines Are Available?

Two vaccines received Emergency Use Authorization (EUA) from FDA, for individuals 16 years of age and older, in December 2020. The first vaccine is from Pfizer-BioNTech and the second is from Moderna. Additional vaccines are under development and are expected to be submitted to FDA in early 2021.

2. Do the Vaccines Work?

Data from clinical trials has shown that both of the authorized vaccines are effective, with efficacy in the 94-95% range. By comparison, the flu (influenza) vaccine has an efficacy rate of approximately 70%, and this is against only one of the many components of flu. The 94-95% effectiveness of the COVID-19 vaccines is represented across different subgroups, ethnic and minority groups, and age groups, including people over the age 65.

FDA and CDC leaders discussed the difficulties of studying the vaccines and efficacy for individual rare diseases, because there is not enough data available due to the small patient groups in our community.

3. Are the Vaccines Safe for People Living with Rare Diseases?

Safety data from the clinical trials has been encouraging. The vaccines were tested in randomized trials with large populations. Side effects have been mostly local to the injection site after the second shot, achiness of joints or muscles, and fevers that generally have gone away in a day or two have been reported. Any severe allergic reaction (which FDA says has occurred in 1 in 100,000 individuals or fewer) may be related to one of the components of vaccine and FDA is looking into these cases. The safety of the vaccine rollout is being watched very closely through the vaccine adverse event monitoring system.

The CDC encourages people living with rare diseases to speak with their health care provider or medical team about taking the vaccine. This is a choice that each rare disease patient needs to make with their individual provider. The CDC's website (["Interim Clinical](#)

[Considerations for Use of mRNA COVID-19 Vaccines Currently Authorized in the United States](#)") offers safety information, with a section dedicated to underlying conditions.

4. When Can I Get the Vaccine?

In the initial vaccine rollout, supply has been limited and is being distributed in phases. Federal health authorities are working with state and local partners to try and get vaccine to as many people as possible, as quickly as possible, and to prioritize vaccines for those at risk for the most severe outcomes. Data has shown that older adults in long-term care facilities are the most vulnerable. At the same time, frontline healthcare workers who are treating COVID patients, and other healthcare workers encountering patients with medical conditions, are being prioritized. This is to protect the most vulnerable and to keep the healthcare system functioning.

Currently, data is limited to support whether patients with a certain rare condition are at risk for more severe outcomes of COVID-19, though CDC recognizes that people who are medically fragile, or who have any diminished ability in lung or neurological function, should be eligible for priority vaccination. The CDC's website ("[People with Certain Medical Conditions](#)") specifically mentions the issue of having insufficient data regarding COVID-19 severity and certain rare diseases, and advises anyone with an underlying medical condition to speak with their medical provider to determine risk factors and whether extra precautions are warranted. NORD has called for people with rare diseases whose health providers believe them to be at higher risk for severe COVID-19 to have access to [vaccines](#) in the same prioritization group as those whose conditions are listed on the CDC's website as putting them at higher risk, and we will continue to advocate on this and [related topics](#).

5. Where Can I Get the Vaccine?

The best place to find information about when and where to get the vaccine is on your state or county health department website. You can find your state health department website by searching online or [through the state health department directory on the CDC's website](#).

6. Can I Choose Which Vaccine to Take?

When the vaccine becomes available to you, you likely will not have a choice due to limited supply. The data shows both vaccines are highly safe and effective. Upon consultation with a physician, patients should feel confident in taking whatever option is first available.

7. Can People Who Are Immunocompromised Take the Vaccine?

The vaccine appears to be safe for people who are immunocompromised. Some people who were immunocompromised participated in the clinical trials. There is still a question as to if the vaccines will be any less effective in people who are immunocompromised. Even if there is a potential loss in efficacy, Dr. Peter Marks, head of the Center for Biologics Evaluation and

Research at FDA, shared his recommendation that the benefits of receiving the vaccine may outweigh any risks.

8. Can Children and Caregivers in the Rare Disease Community Receive the Vaccine?

Pediatric clinical trials for the vaccines are underway. More data is being collected on people aged 12-15. Once safety is confirmed in that age group, researchers will use a step-down approach to study the 7-12 age group. Below 7 years of age, researchers will begin by looking at how the vaccine correlates with protection or antibody responses. The focus of these studies is on the vaccine's safety, confirming the right dose for each age and to ensure that children do not develop inflammatory syndromes or get the virus. The speakers expressed hope in being able to offer vaccines to 12-15-year-olds within a couple of months, and by summer to have data that vaccines can be administered to younger children.

For the parents or caregivers of people with chronic medical conditions, Dr. Amanda Cohn, Deputy Director of Immunization Services Division at CDC, recommended speaking with your healthcare professional to see if you are able to get the vaccine.

9. Can I Receive the Vaccine If I am Undergoing Gene Therapy Treatment or Participating in a Clinical Trial?

The two authorized vaccines have mRNA molecules packaged in something similar to a soap bubble, unlike the vectors being used to administer gene therapies. Because of these differences, no one should be concerned that getting the vaccine would prevent them from being able to receive a gene therapy, according to FDA's Dr. Peter Marks.

If you are taking part in a clinical trial, including a gene therapy trial, you should check with your provider about any potential study protocols before getting the vaccine. Many protocols allow participants to get vaccinated unless there is a specific reason.

10. Are Non-Injection Vaccine Options Available?

For people with certain rare diseases, injections are not feasible. As a result, some people in the rare disease community may not be able to take the vaccine in its current injectable form. For instance, in fibrodysplasia ossificans progressive (FOP), bone growth can develop at the site of the intramuscular injection. Individuals are encouraged to discuss potential benefits and risks of the vaccine with their provider.

On the webinar, Dr. Peter Marks of the FDA shared there are additional vaccines under development that have the potential to be given subcutaneously. Within a few months there should be data on the safety and efficacy on administering vaccines subcutaneously.

11. Do the Vaccines Prevent Transmission?

It is not yet known if the vaccines interrupt transmission of the virus. Animal studies show that the vaccine prevents transmission, but more data is needed from human studies.

12. Should I Get the Vaccine If I Have Had Allergic Reactions to Medicine or Vaccines in the Past?

Individuals who have had an earlier allergic reaction to any medication or vaccine should discuss their history with their medical team, including the healthcare provider administering their vaccine.

Vaccine administration sites must have capabilities to manage allergic reactions. Vaccine recipients will be watched for a certain period of time after receiving the vaccination (usually 15 minutes). Anyone with a history of allergic reactions will need to wait for 30 minutes before leaving the facility so that any reaction can be treated right away.

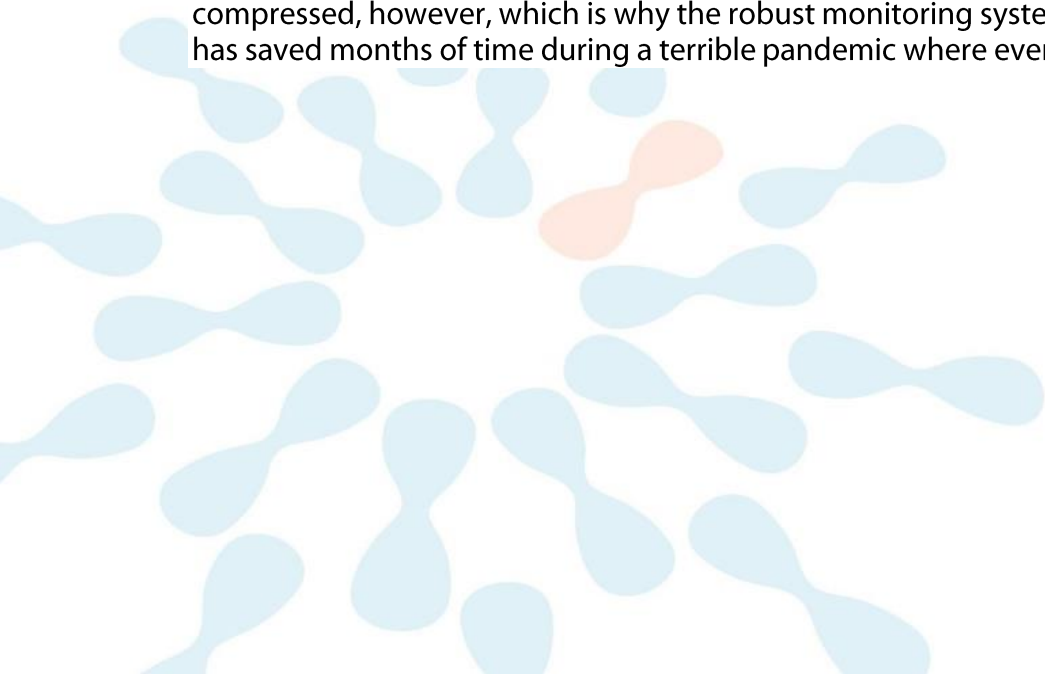
13. Should I Get the Vaccine If I Have/Had COVID?

If you are or were sick with COVID-19, it is not recommended to get the vaccine until you have recovered. The CDC's website (["Interim Clinical Considerations for Use of mRNA COVID-19 Vaccines Currently Authorized in the United States"](#)) offers information on the timing of vaccination post-illness, based on what is known about reinfection rates.

14. How do EUAs Differ from Approvals?

Emergency Use Authorization (EUA) is a regulatory process developed after 9/11 that can be used for products that are meant to address chemical, biological, radiological or nuclear threats. The EUA allows FDA to take a product through an expedited authorization process and make it available to the public for a particular use.

Because vaccines are given to so many people, and because safety is so important, FDA made it clear in their COVID-19 vaccine guidance that the agency would follow the same rigors of a normal vaccine application. The normal one-year follow-up period for the vaccine cannot be compressed, however, which is why the robust monitoring system is in place. This approach has saved months of time during a terrible pandemic where every day counts.



15. Will the Vaccines Protect Against New Strains of the Virus?

FDA and CDC are working closely with federal partners, including NIH, to monitor new strains of the virus very closely. If a new strain arises that is resistant to the current vaccines, they have the pathways to alter the vaccine to provide efficacy against the mutation, and the process will not require another big 4–6-month clinical trial. FDA wants people to know that they are planning and preparing for the unexpected.

