Viltolarsen Factsheet

Information about viltolarsen, the study drug used in the RACER53 study.

What is viltolarsen?

Viltolarsen is an investigational medication given to boys with Duchenne muscular dystrophy (Duchenne) as a weekly, intravenous (IV) infusion that takes 1 hour. It is designed to help the body make dystrophin protein, which is important to muscle development.

Usually, an investigational medication can only be used in research studies because it has not yet been approved as a therapy for a specific condition. **In August 2020, the US Food and Drug Administration (FDA) granted "accelerated approval" to viltolarsen (also called Viltepso) as a therapy for Duchenne that qualifies for exon 53 skipping.** This type of FDA approval provides people with Duchenne with earlier access to viltolarsen while a confirmatory clinical study is ongoing.

Viltolarsen has also received conditional approval in Japan. The "Conditional Early Approval System" is a system to put highly useful and effective drugs for treating serious diseases into practical use as early as possible. This type of approval is granted on the condition that post-marketing surveys or other studies are conducted to re-confirm the safety and efficacy of viltolarsen. Drugs considered for this approval must meet the following conditions:

1. For severe diseases
2. Apparent improvement of medical care
3. Confirmatory clinical trials are difficult to conduct, or do not have sufficient feasibility
4. Confirmation of a certain degree of efficacy and safety through clinical trials other than confirmatory clinical trials

How does viltolarsen work?

People with Duchenne have a mutation (or error) in the genetic code that helps make dystrophin protein. Without functional dystrophin protein, muscles cannot fully develop. Viltolarsen, known as an "exon skipping agent," is designed to "skip" over the incorrect section of genetic code so the body can still make functional dystrophin protein.

Have other people taken viltolarsen?

Viltolarsen has been studied in 42 boys with Duchenne that qualifies for exon 53 skipping as part of clinical research studies in Japan, Canada, and the U.S. Results from these studies included the following:

- An increase in dystrophin protein was observed in study participants who received viltolarsen.
- Study participants who received viltolarsen showed improvement in certain muscle strength and function tests.
- The most common side effects were upper respiratory infection, cough, fever, and injection site reactions.

Where can I learn more about viltolarsen?

To learn more about viltolarsen, visit the following websites:

- viltepso.com
- nspharma.com/pipeline

You can also read a press release about the FDA's accelerated approval of viltolarsen at fda.gov*.

*https://www.fda.gov/news-events/press-announcements/fda-approves-targeted-treatment-rare-duchenne-muscular-dystrophy-mutation