

FACTSHEET

Vyndaqel[®] (tafamidis meglumine) and Vyndamax[™] (tafamidis)

What are Vyndaqel and Vyndamax?

Vyndaqel/Vyndamax are oral TTR stabilizers developed for treating patients with transthyretin amyloidosis (ATTR) who have heart involvement (cardiomyopathy). ATTR amyloidosis is caused by the misfolding and subsequent build-up of a protein called transthyretin – or TTR for short. This build-up of TTR in tissues and organs stops them from working properly. There are two different types of ATTR amyloidosis: one is caused by an inherited mutation, called hereditary ATTR (hATTR), and the other which is not inherited, called wildtype ATTR (ATTRwt).

How do Vyndaqel and Vyndamax work?

Vyndaqel/Vyndamax are drugs that act as TTR stabilizers. Vyndaqel/Vyndamax stabilize TTR by acting as a ‘chaperone molecule’, which means they bind to TTR and help it form correctly. By stabilizing TTR and reducing the rate at which TTR misfolds, Vyndaqel / Vyndamax have the potential to limit amyloid formation and subsequently slow the progression of the disease.

How are Vyndaqel and Vyndamax different?

Vyndaqel is a 20 mg tafamidis meglumine capsule. This was the formulation of the drug used during the ATTR-ACT trial and is recommended as a once-daily dose of 80 mg (four 20 mg capsules). Vyndamax is a free-acid formulation of tafamidis, that was developed for patient convenience. Vyndamax is a once-daily oral capsule (61 mg) which has the same effect as four capsules of Vyndaqel. Vyndaqel and Vyndamax have the same mechanism of action, biologic effect, and are both approved for the treatment of ATTR cardiomyopathy, but are different formulations of tafamidis.

How are Vyndaqel and Vyndamax administered?

Both Vyndaqel and Vyndamax are taken daily as oral capsules.

Clinical trial evidence for Vyndaqel and Vyndamax

In the randomized, double-blind, placebo controlled Transthyretin Amyloid Cardiomyopathy (ATTR-ACT) Phase III clinical trial, 264 ATTR-CM patients received

Vyndaqel (20mg or 80mg) daily for 30 months. Results from the trial have shown that, compared to those on placebo, patients who received Vyndaqel:

- Saw statistically significant reduction in all-cause mortality
- Saw statistically significant reduction in the frequency of cardiac related hospitalizations
- Had significantly greater exercise capacity and quality of life

Side effects of Vyndaqel and Vyndamax

Like all drugs, both Vyndaqel and Vyndamax can cause side effects. The most commonly observed side effects in clinical trials were urinary tract infection, vaginal infection, upper abdominal pain (stomach ache), and diarrhea.

Availability of Vyndaqel and Vyndamax

Vyndaqel and Vyndamax were approved by the FDA on May 6, 2019 for the treatment of ATTR cardiomyopathy (ATTR-CM) in the United States.

Vyndaqel has already been approved and is currently marketed for the treatment of hATTR polyneuropathy in 40 countries, including Japan, Mexico, Argentina, and throughout Europe.

Insurance coverage of Vyndaqel and Vyndamax will vary depending on the particular plan. If insurance does not provide enough coverage, there are additional programs that can be looked in to. This includes Pfizer's patient support program, VyndaLink. You can learn more about this program through the following link: <https://www.vyndalink.com/>

More information

For more information you can visit us at www.arci.org. If you have further questions you can contact us by phone at 617-467-5170 or by email at arc@arci.org.