

News Alert

September 26, 2023

Ionis - positive phase 3 study Olezarsen

Aescap portfolio company Ionis announced today that its medicine candidate Olezarsen has shown a statistically significant reduction in triglyceride levels in a phase 3 clinical study. High triglycerides can cause cardiovascular events like heart attacks and strokes.

The study was performed in patients with familial chylomicronemia syndrome (FCS) and met its primary endpoint: a statistically significant reduction in triglyceride levels versus placebo. Olezarsen also resulted in a 100% reduction of acute pancreatitis events compared to placebo, a key secondary endpoint. Next to being effective, Olezarsen demonstrated it is safe and well tolerated.

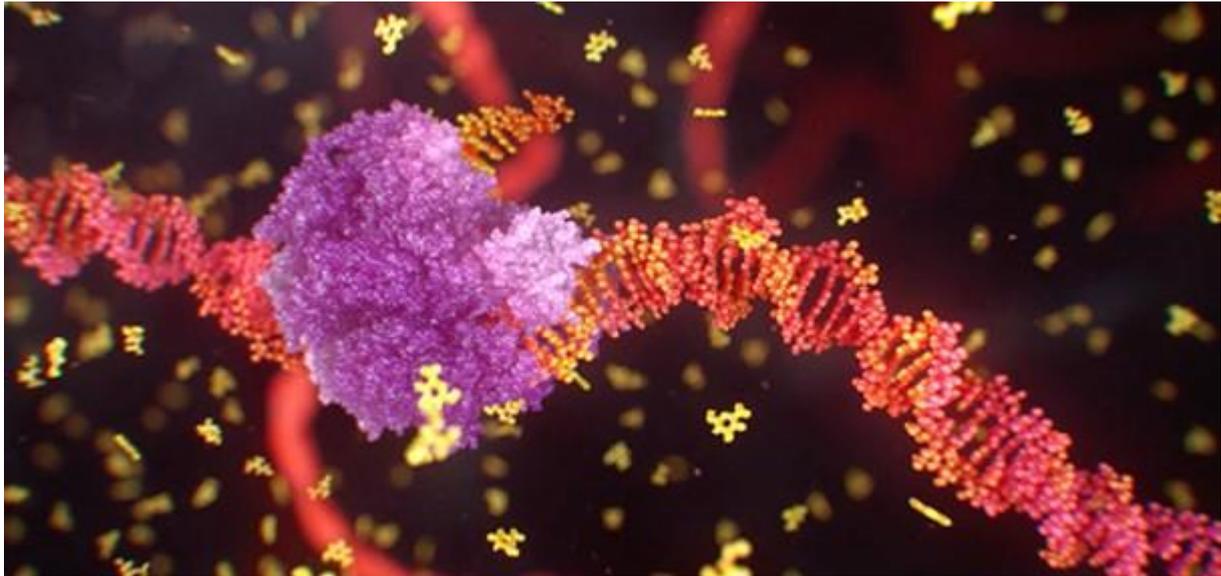
Ionis is preparing for the approval process and launch of the medicine. So far, no medicine for the treatment of FCS is approved.

Ionis is part of both the Aescap Life Sciences fund (7%), and the Aescap Genetics fund (11%).

Hidden Potential Value

Although FCS is a rare disease and therefore the value of the medicine in terms of revenues is limited, the study results pave the way to a much larger market potential. Given the triglyceride reduction Olezarsen is generating, it can most likely be of use for the treatment of people that suffer from severe hypertriglyceridemia (SHTG).

In the Western world only, over 5 million people have this 'condition'. Phase 3 studies in this larger patient population are in progress and will read-out in 2024.



DNA to RNA transcription proces

Why Ionis so often appears in our newsletters

You may have noticed that Ionis is often mentioned in our newsletters. The reason is that the company's RNA-targeted medicines have the potential to treat many diseases where other therapeutic approaches have proven ineffective or do not exist.

Ionis has 26 medicine candidates in development that are tested in clinical studies which are generating regular positive results. This increases the company's future earning power.

The company has four products on the market so far. These are marketed by Biogen SOBI and PTC. In 2022, Ionis received \$303 million in royalties and \$284 million in milestone payments.

Latest December 22, Ionis is expecting its next medicine, Eplontersen, to be approved. We think the product has an annual sales potential of around one billion euro. It will be the first product the company is going to market in the US themselves, although in tandem with multinational AstraZeneca that is also responsible for sales outside of the US.

Olezarsen, is planned to be marketed by Ionis independently. This allows the company to book 100% of sales instead of the approximately 10-20% royalties plus milestone payments it receives when it out-licenses products.

About FCS and Olezarsen

“These positive Olezarsen results represent an important advance for people with FCS who live in constant fear of unpredictable and potentially fatal attacks of acute pancreatitis. With no currently FDA-approved treatments, people with FCS live with debilitating abdominal pain and must maintain an extremely restrictive diet consisting of less than 20 grams of fat per day,” said Sam Tsimikas, M.D., senior vice president, global cardiovascular development at Ionis.

"In this study, people with FCS treated with Olezarsen along with background therapy and a low-fat diet had a substantially reduced risk of recurrent attacks of acute pancreatitis. These results strengthen our confidence in Olezarsen's potential to deliver benefits to FCS patients and in the larger population with SHTG following completion of ongoing Phase 3 studies.”

In Q1 2023 the FDA granted Olezarsen Fast Track designation for the treatment of FCS which is helpful in the approval process, and which was probably driven by the fact that no treatments are available so far.