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Ikaria's new CHF drug is really . . . a device?

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If it looks like a drug, and acts like a drug, then it must be a . . . device. It might sound strange but that's exactly the case for **Ikaria**'s (Hampton, New Jersey) IK-5001, a therapy administered by injection to support the heart muscle.

The product is geared toward treating patients who have suffered from a heart attack and could be at risk to develop congestive heart failure.

"The great unmet need in managing myocardial infarction is how to prevent this progression of ventricular remodeling [which can lead to] congestive heart failure," Daniel Tasse, Ikaria's CEO told *Medical Device Daily*. "That's what this product does and it's pretty clever how it does it."

Tasse said once the heart is damaged from a heart attack then it releases calcium. What happens then, is that the physician injects about 2 ml of the solution into the patient.

Then according to Tasse, when the IK-5001 solution meets with calcium in the heart muscle, it turns from a liquid to a gel like substance, and acts like a cast supporting the heart muscle. When the calcium levels decrease in the heart, the "cast" goes back to a liquid state and is excreted through the Kidney

"That splint has the same elastic properties as healthy heart muscle," Tasse told *MDD*. "So it gives a little bit. It allows the heart to keep on beating."

When Ikaria spoke with the FDA about the best way to classify the product, it said that the FDA agreed with its position that the IK-5001 acts more like a device.

"It has no physiological affects at the cellular level and on top of that it's there when you need it but after six or seven weeks, it's gone," he said.

IK-5001 was licensed in 2008 from the Israeli firm **BioLineRx** (Jerusalem).

The product has not yet received FDA approval and is subject to be evaluated in an upcoming clinical trial. Ikaria said if the product gains approval then it would be classified as a device, and that has definite benefits for the patients and the firm.

"The beauty of the device pathway for patients as well as Ikaria, is that you can demonstrate efficacy with functional endpoints and that was the very rich conclusion we came to in discussions with the FDA," he said. "They have agreed to the endpoints for the development of IK-5001."

The first endpoint would be the measurement of injection fractions. Radiological procedures would be used to show that the heart did not distend as much as it would if a placebo was used. The second endpoint is that a patient a year after their heart attack could walk a longer distance in six minutes than a patient could getting a placebo. The third measurement would assess the patient's quality of life using the product vs. a placebo.

"This can be demonstrated with about 1,500 patients – maybe 2,000," Tasse said. "If you went the drug pathway, you would require 10,000 patients in a five year follow-up."

A clinical trial regarding the product could be launched in the next month or two, but definitely before the end of this year, the firm said. A phase III evaluation of the product will follow.

"If all goes as planned the product should be launched in early 2014 or 2015," he said.

Ikaria was formed in March of 2007 when the company merged with **INO Therapeutics**. It acquired INO for $670 million in a cash and stock merger. Previously the company had about 300 employees, half of which were based in New Jersey. Currently the company has 500 employees; 300 in New Jersey.

The INO acquisition is significant because with it came Inomax, a treatment for infants born with respiratory distress. According to Tasse the product generated nearly $300 million in sales and helped fund a good portion of the company's R&D efforts.

If IK-5001 garners FDA approval it could have the potential to impact a huge market, and that's what the company is counting on.

"The market potential is huge," Tasse said. "There are about one million heart attacks in the U.S. every year and about 300,000 in Europe. Somewhere between 40 to 60% of those patients will have a kind of massive heart attack . . . where they are at risk for developing congestive heart failure four years later. So you can see the market is quite big."