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Drug Firms Buy $67.5 Million Voucher to Speed FDA Review

Regeneron, Sanofi Hope Voucher Will Help Them Outflank Amgen in Race to Get New Cholesterol Drug to Market

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Regeneron Pharmaceuticals Inc. REGN +0.05% and Sanofi SA SAN.FR +0.58% are spending $67.5 million on a novel bet they hope will help them outflank Amgen Inc.AMGN +0.10% in the race to get a new class of cholesterol drugs to the market.

The companies are paying the money to acquire a special voucher held by BioMarin Pharmaceuticals Inc. in a bid to hasten regulatory review of their drug alirocumab, one of an emerging group of medicines that lower cholesterol by targeting a gene known as PCSK9.

BioMarin was awarded the voucher early this year as part of an incentive program established by the U.S. Food and Drug Administration to encourage development of drugs for rare pediatric diseases. The voucher entitles the holder to ask the FDA for priority review of a drug application that would otherwise get a standard review. That could shorten the review process to six months from the standard 10 months.

BioMarin received the voucher in conjunction with FDA approval of Vimizim, a treatment for a rare pediatric condition called Morquio A syndrome that afflicts about 800 patients in the U.S. While BioMarin could have used the voucher itself, the program also allows companies to sell a voucher to another company. The voucher doesn't need to be used on a drug for a rare pediatric condition.

The voucher was the first to be issued under the pediatric incentive program, and also the first to change hands. A separate program to encourage development of medicines for rare tropical diseases already existed.

Regeneron says it sees the voucher as a way to potentially get alirocumab to the market faster—and to pull even with or even overtake Amgen, whose anti-PCSK9 agent is called evolocumab.

Regeneron, in partnership with Sanofi, was the first company to show a PSCK9 inhibitor lowered cholesterol in people and to report a successful late-stage study, Regeneron Chief Executive Officer Leonard S. Schleifer said. But Amgen "got ahead of us, apparently."

Amgen said Tuesday it plans to file its drug application with U.S. and European regulators this quarter while Regeneron and Sanofi expect to file by the end of the year.

Amgen didn't have any further comment.

The voucher could close any advantage Amgen may have in advancing toward the market or perhaps put Regeneron and Sanofi back in the lead, Dr. Schleifer suggested. Regardless, "it could potentially shorten the time of the drug getting to patients by about four months."

A chess player since childhood, Dr. Schleifer described the PCSK9 race to market as an "interesting chess game." He said Amgen pulled ahead by running trials that tracked patients for 12 weeks compared with 24 weeks in the Regeneron/Sanofi studies. Now, with the unusual purchase of the voucher, he said, "Let's say we came up with a discovered 'check' here."

Regeneron, along with Sanofi, and BioMarin separately announced the deal for the voucher on a day when Regeneron and Sanofi also disclosed that alirocumab successfully achieved significant cholesterol reduction in nine late-stage trials.

Whether the voucher will lead to faster approval of the drug isn't certain.

"It's going to be tough to speculate," Christopher Viehbacher, chief executive of Sanofi, said in an interview. But data from the nine studies "gives us a lot of confidence in the approvability of the drug."
The companies released few details of the data, which are expected to be released in full at upcoming medical meetings, including the European Society of Cardiology congress in Barcelona at the end of August.

For the PCSK9 drugs to reach their full commercial potential, companies will have to prove the treatments reduce long-term health problems associated with high cholesterol, analysts said.

Regeneron and Sanofi said in a news release the nine studies met their primary endpoints for lowering patients' LDL-cholesterol, sometimes called bad cholesterol, compared with patients receiving placebo or an active drug such as ezetimibe, sold as Zetia by Merck MRK -0.20% & Co.
Among the most common adverse events in the studies were upper respiratory infections, which were balanced between patient groups, the companies said. Serious adverse events and deaths were generally balanced between patients taking the drug and patients receiving placebo or an active comparator drug.

The companies also said they observed a positive signal in one of the late-stage studies in which patients taking alirocumab had fewer major heart problems such as stroke and heart attack compared with patients receiving placebo. Regeneron's Dr. Schleifer said this finding is of limited value because it wasn't part of the trial's prespecified endpoints, but is an encouraging sign the drug could show long-term health benefits in an 18,000-person study the companies are conducting.

"It's not formal scientific proof of anything, but it helps us think the hypothesis we're testing is a good one," Dr. Schleifer said.

Unlike many current cholesterol-lowering therapies, which are sold as pills, PCSK9 treatments must be injected, making them potentially less convenient for many patients. The lower convenience of the PCSK9 drug also raises the bar for the level of efficacy many patients and health insurers will want to see from the drug before paying for them, doctors said.

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