ENDPOINTS NEWS

Sangamo to seek accelerated approval for Fabry gene therapy

by **Max Gelman on October 22nd, 2024**

Sangamo Therapeutics thinks it's found a faster, earlier pathway to approval for its Fabry disease gene therapy.

The biotech announced Tuesday that it plans to seek an accelerated approval from the FDA for the gene therapy. And the company's executives told *Endpoints News* that the FDA won't require them to run a separate confirmatory study should it be approved.

The FDA has signaled more openness toward accelerated approvals, particularly in rare disease and cell and gene therapies. Sangamo CEO Sandy Macrae told *Endpoints News* in an interview that the FDA has not asked for anything outside of the data Sangamo already submitted, and that longer-term data from the existing trial might be sufficient as a follow-up.

Sandy Macrae

“One thing they may ask for is that we continue the trial, so everyone gets to two years and that we submit that two-year data,” Macrae said. “They have not even asked for that. There is no ask from the FDA, and the language in the letter is black-and-white clear. There is no requirement for any additional trial.”

Those two-year data could come up again in discussion during Sangamo’s BLA meeting with the FDA, which is likely to be in the second half of 2025 before the company files for approval, he added.

“We remain committed to discussing trial designs with applicants and considering all our statutory authorities and any flexibilities provided by those authorities, including the use of accelerated approval, as appropriate, for the rare disease under consideration to help advance development,” an FDA spokesperson said.

One implication of not running a confirmatory trial is that an accelerated approval wouldn’t likely be converted to a full approval, which could affect insurance coverage or the drug’s pricing.

Wilson Bryan, the now-retired director of the office that approved cell and gene therapies, previously expressed concerns that confirmatory studies could take years to confirm benefit in the cell and gene space, or be infeasible if the populations are too small.

CBER Director Peter Marks said in February that he wants accelerated approval to "be the norm" for gene therapy, and that using biomarkers yet to be qualified won't be much of a hurdle.

Sangamo said Tuesday that it intends to use data from a Phase 1/2 study to seek the approval, using "eGFR slope at 52 weeks across all patients" as the surrogate endpoint.

At that point, researchers will have one-year data from all 32 patients in the study and two-year results from 19 of them, Macrae said.

Company struggles

Sangamo has faced significant setbacks in recent months, with its share price down roughly 95% from its 2020 peak before Tuesday. Its stock $SGMO closed up 32%.

Since April 2023, Sangamo has laid off a majority of its workforce, letting go 360 employees across three restructurings. It's also struggled to raise cash — according to an SEC filing recapping its second-quarter earnings, Sangamo only has enough runway to last into the first quarter of 2025.

But Macrae said Sangamo's finances were not the reason the company decided to seek accelerated approval. Sangamo is engaged in the "late phase" of a number of different partnership opportunities to try to raise some cash, Macrae said, and he said Tuesday's news would only boost those efforts.

"I know that people have worried about our finances," Macrae said. "We, too, in the company, have worried about them, and until we can bring in that non-dilutive funding — which we now believe is in sight — we will not get the appropriate shareholder value, and that bothers us and is the focus of what we've been trying to do."

Louise Wilkie, Sangamo's head of corporate communications and investor relations, said there's no timeline for when those partnerships might happen.

In August, Sangamo partnered with Roche's Genentech on a tau-lowering program for $50 million upfront and $1.9 billion in potential milestones.

Editor's note: This story has been corrected to clarify that Sangamo would be allowed to use an extension of an existing study as a confirmatory trial. A comment from the FDA was also added.

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