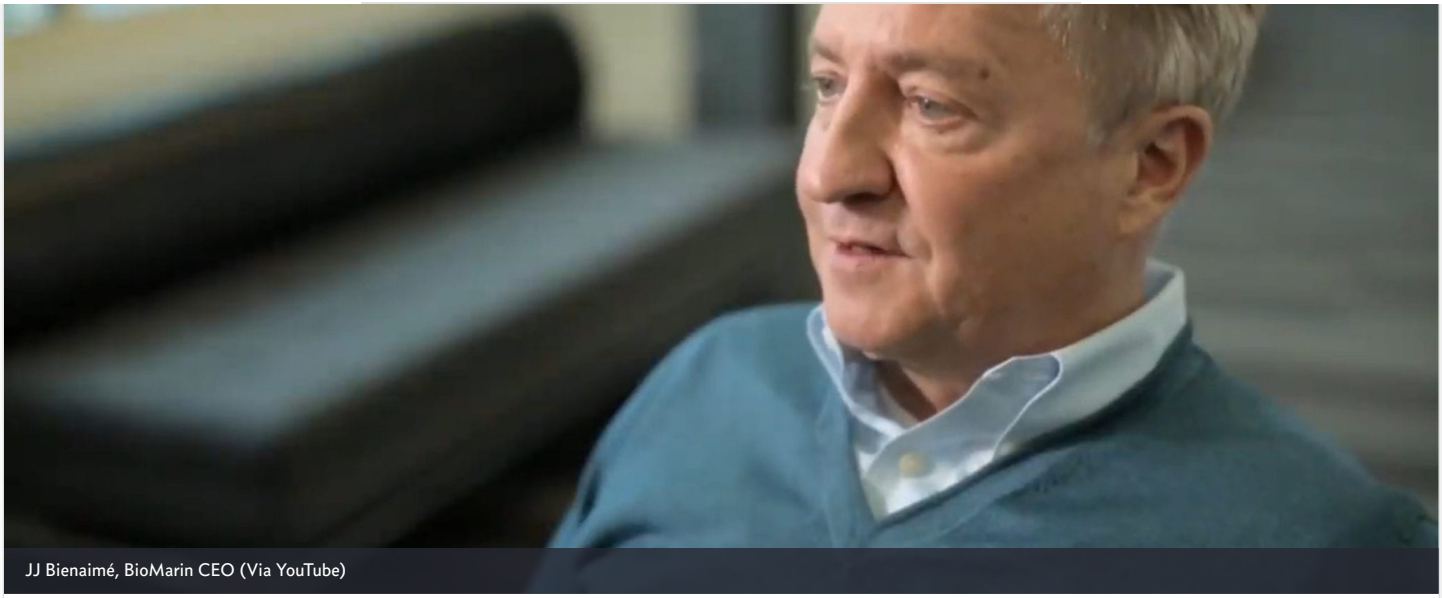

JJ Bienaimé, BioMarin CEO (Via YouTube)

July 22, 2021 01:48 PM EDT
R&D

New data show BioMarin's troubled hemophilia gene therapy continues to fade, opening room for rivals

Jason Mast
Editor

The effects of BioMarin's vaunted gene therapy for hemophilia A are continuing to fade in the earliest treated patients, renewing questions about the long-term prospects for a drug the company had billed as a one-time cure.

At the International Society on Thrombosis and Haemostasis meeting Wednesday, BioMarin announced that, after five years, the first seven patients treated with the therapy's high dose expressed a median of just 8.2% the amount of factor VIII that a healthy person would. That's a small fraction of the 60.3% expression patients saw one year after receiving the therapy, known as valrox.

BioMarin highlighted — as they have before — that despite the waning levels of protein, patients were still largely symptom-free. The therapy reduced the annualized bleeding rate by 95% among six of the patients, and even in year 5, six of the seven patients had no bleeding events at all.

sickle cell and many genetic diseases now being targeted by gene therapies, researchers don't actually have to get patients to complete normal expression in order to alleviate symptoms and allow people to lead healthy lives; often a fraction will do.

Precisely how large a fraction, though, remains unknown, and BioMarin's dwindling levels have raised flags with regulators. Last year, in a surprise move, the FDA rejected the company's application for approval, saying they would need to see more durability data before allowing BioMarin to put the drug on the market.

Durability is a greater concern with modern gene therapies than it is for other medicines. Patients can only be dosed once, giving researchers only a single shot to cure the disease. And the company's CEO JJ Bienaimé has promised to price valrox as if it were a cure: between $2 million and $3 million per year.

The new data, though, show that gene expression has steadily declined year-to-year, underscoring concerns that it could one day drop below levels that are therapeutically relevant and keep patients off of transfusions.

Those fears may not prevent BioMarin from obtaining approval. The company expects to have two-year data from its Phase III trial in early 2022, at which point they would likely refile with the FDA.

It could, however, drastically diminish the therapy's benefit for patients and how much money BioMarin can make. With other hemophilia A gene therapies also nearing approval, patients may wait for something that looks closer to a one-time cure.

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August 31, 2021 10:34 AM EDT
Updated 11:11 AM
People, FDA+

BREAKING: In a major blow to vaccine efforts, senior FDA leaders stepping down

Zachary Brennan
Senior Editor

Marion Gruber, director of the FDA's Office of Vaccines Research & Review and 32-year veteran of the agency, will leave at the end of October, and OVRR deputy director Phil Krause, who's been at FDA for more than a decade, will leave in November. The news, first reported by BioCentury, is a massive blow to confidence in the agency's ability to regulate vaccines.

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Endpoints Staff

Biopharma is no stranger to major scientific innovations, but when you talk to the movers and shakers in the field on who has influenced their work the most, you start to hear a few key names over and over again.

That was certainly the case when we polled industry executives on who they viewed as the R&D luminaries of note — men and women across the drug development spectrum who take major biological and chemical breakthroughs and spin them like gold into medical miracles. These are the people who opened doors to new fields of drug research, laying the foundation for much of the work now underway in the clinic.

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August 31, 2021 11:05 AM EDT
Updated 01:59 PM
R&D

UPDATED: Cautiously optimistic, AC Immune delivers a mixed set of Alzheimer's data — but investors love it

Swiss biotech AC Immune is trading sharply higher Tuesday morning following the release of mixed topline Alzheimer's data.

First, the good news: In a double-blinded and randomized Phase II study, AC Immune's tau-targeting monoclonal antibody met a primary endpoint showing the candidate slowed cognitive decline at a statistically significant rate in mild-to-moderate

August 31, 2021 10:02 AM EDT
Updated 10:34 AM
R&D, Cell/Gene Tx

Moving past a patient death, Poseida offers an early snapshot of proof of efficacy for a next-gen solid tumor CAR-T

John Carroll
Editor & Founder

Ten months after the FDA took the shackles off Poseida's study for a CAR-T directed against castrate-resistant prostate cancer, the executive team is now rolling out an early snapshot of the proof-of-concept data it's looking for in a small, early-stage study.

Tested in 9 heavily pre-treated patients — with an average of 6 prior therapies — investigators tracked a significant, 50%-plus drop in PSA levels in 3 of those patients and what they called a "concordant" imaging result for tumor effect. One patient had a complete response — the ideal result for any cancer study.

August 30, 2021 11:00 AM EDT
Updated 04:40 PM
Discovery, Cell/Gene Tx

August 30, 2021 06:30 AM EDT
Updated 13 hours ago
Financing, Cell/Gene Tx

Big money: Flagship's uber-platform play Laronde rakes in $440M to chase programmable 'eRNA'

Kyle Blankenship
Managing Editor

With the success of Moderna, parent company Flagship Pioneering has become the mothership for even more audacious next-gen platform plays. Investors like what they see, and now they've opened the vault to a particularly bold Flagship startup working on ring-shaped RNA it thinks could be the future of drugmaking.

Laronde has closed a $440 million Series B — one of the largest ever fundraising rounds of its type — to pursue a programmable RNA platform the biotech has previously said could churn out 100 marketed drugs or drug programs in 10 years.

August 31, 2021 07:25 AM EDT
R&D

August 31, 2021 07:00 AM EDT
Updated 07:29 AM
Financing, Startups

A Novartis castoff drug gets new life at an Atlas-backed startup looking to blaze a trail in obesity

Kyle Blankenship
Managing Editor

After decades of failure, the obesity field is finally seeing some major progress with the success of a stable of GLP-1 diabetes drugs showing clinical benefit. A small biotech thinks it has something new to offer in that space with an older drug, and investors like what they see so far.

Versanis Bio launched Tuesday with a $70 million A round backed by biotech blue-chippers Atlas Venture and Medicxi with lead candidate bimagrumab, an in-licensed Novartis drug originally targeting muscle weakness, gearing up for a Phase II study in obesity.

FDA extends full approval for Merck's Keytruda in 1st-line bladder cancer after mixed adcomm vote

Kyle Blankenship
Managing Editor

Merck's accelerated approval for Keytruda in first-line bladder cancer was one of a slate of early nods put under the microscope by an FDA advisory committee earlier this year after flopping confirmatory tests. Keytruda passed that test, and now the FDA is making its accelerated OK official.

The FDA on Tuesday gave its full approval to Keytruda as a treatment for first-line advanced bladder cancer patients who are not eligible for platinum-based chemo despite a confirmatory outcomes trial in an expanded patient population turning up a dud, Merck said in a statement.

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