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For Cahill Atty, Rare Disease Pro Bono Work Is Personal

By **Mark Payne**

Law360 (September 16, 2025, 3:12 PM EDT) -- New York attorney John MacGregor was on the phone with his father as he commuted home from his Manhattan office when he shared the news.

John MacGregor's son, also named John, during his first hospital stay, then aged 10 months. (Courtesy of John MacGregor)

His wife, Sally, had just told MacGregor that their then 10-month-old son, also named John, had a seizure. MacGregor's father, a physician, asked how long the seizure lasted.

Fourteen minutes, MagGregor told him. His father paused.

"Are you sure she didn't mean seconds?" he asked.

Two weeks later, John's son had an even longer seizure, lasting 20 grueling minutes. Three weeks after that, he suffered a seizure that lasted a full hour. Initial tests didn't reveal a cause.

"Everything was normal," MacGregor, 38, told Law360. "It's perplexing why somebody would be having seizures this long and this intense."

Eventually, doctors diagnosed the baby with Dravet syndrome, a rare type of severe epilepsy. Dravet typically presents in infants and causes debilitating seizures that can lead to delays in development or even premature death. It affects roughly one in every 15,550 children.

It was the beginning of a long journey for MacGregor and his family. Today, MacGregor, a litigation partner at Cahill Gordon & Reindel LLP, is representing pro bono a foundation dedicated to curing his son's disease, hoping to make it easier for people with rare diseases to access new drugs.

"I know firsthand all these barriers to access that rare disease patients have to overcome in order to get these gene therapies," said MacGregor, who had no previous healthcare experience.

'Shock'

The MacGregors were in shock when they learned of young John's diagnosis.

"I didn't know anything about rare diseases," MacGregor told Law360, but he said he was "action-oriented" after learning of the diagnosis and getting a disease "welcome packet" from the Dravet Syndrome Foundation.

He presumed at the time that with a genetic disease like Dravet, which is caused by a mutation in the SCN1A gene, there would be a gene therapy available that helps cure the underlying cause of the disease. He was wrong.

Access to gene therapies is complicated. There are at least 10,000 rare diseases, but so far, there are only 46 gene therapies approved by the U.S. Food and Drug Administration. The process to get a drug to market is time-consuming, complicated and expensive.

Congress sought to help patients overcome this hurdle in 1983 by passing the Orphan Drug Act, which has helped bring 882 FDA-approved orphan drugs to market for 392 rare diseases. Most of those drugs only treat symptoms.

Scientists, advocates and others are hoping that technological advancements in gene editing will help bring drugs that can address the root causes of the disease, primarily by making changes to a patient's DNA.

The process to get approval for gene therapies starts once a drug company has industry backing. Drugmakers must file an investigational new drug, or IND, application to the FDA detailing the drug's preclinical efficacy in cell and animal testing and show how they plan to test in humans.

If approved for an IND, drugmakers start Phase 1 of clinical trials, in which they test it out on a small group of humans. In Phase 2, manufacturers can test it on a larger pool of patients.

After Phase 2, drugmakers discuss the safety and efficacy results with the FDA before moving on to Phase 3 with a larger patient population.

If Phase 3 is successful, drugmakers can submit a new drug application, or NDA, along with all the data and detail how the drug will be manufactured. If the FDA approves the NDA, then drugmakers can sell the drug on the market.

The process can take between 10 and 15 years and cost billions of dollars, according to a 2023 article in Therapeutic Advances in Rare Diseases. Less than 12% of drugmakers with candidates for rare diseases get their drugs to the market.

Mary Anne Meskis co-founded the Dravet Syndrome Foundation in 2009 after her 4-year-old son was diagnosed. The disease, she said, is a daily burden that affects her entire family.

"We knew there was something happening, but we didn't know that this was going to be a lifelong condition and what the impact would be around him over time," she said.

She was always hopeful that something would come along to help, but didn't anticipate "that there could be something that would be disease-modifying in my son's lifetime."

There are now two disease-modifying therapies currently in clinical trials, and three drugs already approved that help alleviate symptoms.

One drug by Stoke Therapeutics entered Phase 3 trials this year. Meskis is hopeful that if approved, the drug could provide a breakthrough for her son, who needs around-the-clock care. Another drug by Encoded Therapeutics is being enrolled for Phase 2 trials.

"Some of the initial things that we're hearing about the Stoke trial could give him a certain level of independence, like being able to button a shirt or to be able to engage in conversation, would be really meaningful for us," she said.

If the Stoke drug makes it through the Phase 3 trial, it will still need to get NDA approval from the FDA.

Veronica Hood, chief scientific officer at the foundation, told Law360 that it's been a long journey over the last five years as the Stoke drug has worked its way through clinical trials.

"It's been painstaking to watch families who helped make this progress watch as the time slowly ticks by," she said. "Many of the people who helped this progress happen have children who may be too old to access these therapies now that they're being created."

Rare Disease Barriers

While Dravet syndrome advocates wait for a potential disease-modifying drug, the foundation is closely watching an ongoing court case involving another rare disease.

Working pro bono, MacGregor filed an amicus brief on behalf of the foundation in August in a case brought by Massachusetts-headquartered Vertex Pharmaceuticals. It was the foundation's first foray into advocating in the courtroom.

The Vertex suit challenged a U.S. Department of Health and Human Services advisory opinion that a Vertex fertility preservation program, meant to be offered in tandem with a gene therapy to treat sickle cell disease and another red blood cell disorder, could violate federal anti-kickback laws.

Drug manufacturers are not allowed to provide any type of "renumeration" to influence patients to take their drugs if Medicare or Medicaid is footing the bill.

Patients receiving Vertex's sickle cell drug Casgevy have to undergo chemotherapy before treatment, which can affect fertility.

John, upper left, plays with his siblings in August. He's now nearly 3 years old. (Courtesy of John MacGregor)

The proposed Vertex fertility program would provide up to $70,000 to help people using Casgevy with their preservation efforts, including freezing eggs, a relatively expensive step not generally covered by federal insurance programs.

In an advisory opinion, the HHS's Office of Inspector General said the fertility program would qualify as "prohibited renumeration" under federal anti-kickback laws and lead to potential sanctions.

Casgevy is one of the 46 gene therapies approved by the FDA.

In the advisory opinion, the HHS-OIG noted that as more gene therapies come to the market, there will be more potential unknown side effects that require special arrangements, such as specialized care for infertility.

"The number of cell and gene therapies that are available in the marketplace is rapidly increasing; payors, clinicians, and other stakeholders are adapting to the proliferation of these innovative therapies (e.g., paying for and providing these treatments)," HHS-OIG said.

"These treatments are novel, and much is yet unknown about them and optimal arrangements for ensuring appropriate access to them. This uncertainty makes it difficult to assess the risk of the proposed arrangement and offer prospective immunity under our fraud and abuse authorities."

A federal judge in Washington, D.C., handed Vertex a loss in March, ruling in favor of HHS.

Vertex has appealed the case to the D.C. Circuit Court of Appeals.

In its August brief, the Dravet Syndrome Foundation argued that the federal government is "disincentivizing" biotech companies from investing in treatments for people with rare diseases, and that another ruling in favor of HHS could further stymie access to rare disease drugs.

"We thought that this case would be a good place to start with that type of advocacy, because the case seems like it may be limited to a fertility program for a specific disease, but it's really more broadly about access to gene therapies," MacGregor said.

Other advocacy groups, too, see the Vertex case as crucial to rare drug access because the advisory opinion acts as a deterrent for drug development.

Michael. S. Labson, a partner at Covington & Burling LLP, who is representing the Sickle Cell Reproductive Health Education Directive as an amicus in the Vertex case, told Law360 that rare diseases have smaller patient populations and thus far fewer resources available to them, including patient support programs.

"In the case of a rare disease, with patient populations that have burdens, if the manufacturers are able to provide reasonable assistance programs, that can be a real help to patients and get access to important therapies," he said.

The Dravet Syndrome Foundation's Hood said the process to get gene therapies is already so hard, and that a final ruling against Vertex would cement a hurdle for patients trying to access rare disease drugs.

"For how long it's taken to get there — for the money, the time, the investment from companies, from patients and families — I think it's just so important to us that there's not an additional barrier after you meet all of those checkpoints," Hood said.

'All Aspects of Your Life'

MacGregor's son, now nearly 3 years old, is now receiving the best care available, his father said. While the seizures are being treated, the family is preparing for the other comorbidities that come along with

Dravet, such as delayed speech and intellectual disability.

More than 80% of Dravet patients have extensive issues with learning disabilities, leading them to live with their parents or in special healthcare facilities, according to the Dravet Syndrome Foundation. Around 15% die from disease complications when they're children.

Even at such a young age, it's clear that John's twin sister is significantly more advanced than he is, MacGregor said.

"It basically affects all aspects of your life," MacGregor said.

The search for a genetic cure for Dravet syndrome is still at an early stage and rife with challenges.

Most patients have a mutation of the same SCN1A gene, but it's a huge gene, and it has different parts that can get disrupted, Hood told Law360. It's not simply fixing one spot or sending in a new copy of a small gene.

"It's too big for a lot of the current gene therapy packaging, and if you wanted to make a correction in it, you would have to develop a unique therapy for almost every patient with the technology we have right now," Hood said. "So it's a very challenging disease to create a genetic therapy for."

For now, MacGregor said he's watching for other legal advocacy opportunities for the foundation, which he said is open to weighing in.

"We are just keeping our ears open in case anything comes up that might align with our interests," he said.

He and Cahill have been handling some "ad hoc" corporate governance requests for the foundation. Miles Wiley, one of Cahill's partners in charge of the firm's pro bono program, told Law360 that the firm is happy to support attorneys who bring personal passion projects to work on, noting MacGregor's work with the Dravet Syndrome Foundation.

"We're really proud of how our work is making a substantial difference in people's lives," Wiley said.

--Editing by Haylee Pearl.
