**He Designed Drugs to Save His Children. Now He’s Working to Save Biotech, Too.**

## John Crowley will become a top lobbyist for the industry as it confronts investor skepticism and regulatory challenges

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John Crowley burst into the back hallway of a Cheesecake Factory in New Jersey where [his daughter Megan](https://www.wsj.com/articles/doctors-said-shed-die-before-high-school-but-she-wanted-to-go-to-college-11559386800) and her nurse had just finished lunch.

Megan, 26, was lying unconscious on the tile floor next to her wheelchair. She was grayish, her lips purple. Nearly a quarter-century after plunging into biotech to find drugs to save Megan and her younger brother, Patrick, from a rare and deadly genetic disease, Crowley feared the battle had suddenly been lost.

A police officer performed CPR and got Megan’s pulse back. Crowley grabbed a manual breathing bag and started givingMegan air the way he knew works best for her.

A day later, Megan was home recovering from her most life-threatening emergencysince she had pneumonia as a baby. The scare jolted Crowley toward a realization: As far as [rare-disease drugs](https://www.wsj.com/articles/fda-widens-path-for-rare-disease-treatments-with-new-approval-1ba99c09) have come, [they need to be much better](https://www.wsj.com/articles/these-drugs-are-so-futuristic-that-doctors-need-new-training-d88c10dc). He resolved to devote the next act of his career to saving not just his children but the biotech industry.

“There is massive unmet need, and the whole ecosystem just isn’t coming together,” said Crowley, 56, executive chairman of [Amicus Therapeutics](https://www.wsj.com/market-data/quotes/FOLD).

He helped develop [two drugs to treat Megan](https://www.wsj.com/articles/agonizing-choices-for-lives-saved-by-miracle-drugs-1390618942?mod=article_inline), Patrick and others with Pompe disease, which causes heart and skeletal muscles to waste away. The Food and Drug Administration [approved the second drug](https://www.pharmaceutical-technology.com/news/amicus-fda-pompe-disease-therapy/#:~:text=The%20FDA%20has%20approved%20the,Phase%20III%20PROPEL%20pivotal%20study.&text=The%20two%2Dcomponent%20therapy%20is,with%20late%2Donset%20Pompe%20disease.) in September.

Crowley will take on his biggest role yet in March, when he becomes chief executive officer of Biotechnology Innovation Organization, a powerful trade group whose members are mostly small health-focused companies. Crowley will succeed Rachel King, who has served as interim CEO since Dr. Michelle McMurry-Heath [resigned after disagreements](https://www.wsj.com/articles/bio-chief-executive-michelle-mcmurry-heath-exits-after-clashes-with-board-11665444869) with some [BIO board members](https://www.wsj.com/articles/ceo-of-biotech-lobbying-group-bio-on-leave-amid-clash-over-direction-11665325823).

Board members had just met in Washington, D.C., in October and interviewed candidates for permanent CEO when Crowley, the board’s vice chair, broached the idea of applying for the job. It was three days after Megan’s collapseand a day after meeting with patients and advocates at a conference held by BIO.

“I used to say I have two hats. I have my biotech CEO or entrepreneur hat, and I have my dad hat,” Crowley had told attendees of the conference at a Washington hotel. “And then I realized it’s really just one great big hat.”

BIO’s board chair, Dr. Ted Love, said Crowley stood out for his experience as a biotech CEO as well as the breadth of his connections with lawmakers, executives and patients. “John is a guy who can help us change the narrative about the industry,” Love said.

The Wall Street Journal [chronicled the early years](https://www.wsj.com/articles/SB99644757110000000) of Crowley’s work on [the first drug](https://www.wsj.com/articles/SB106184568337857300), which he credits with fixing his children’s hearts, the most life-threatening aspect of the disease. Reporter Geeta Anand expanded on her stories in [a book, “The Cure,”](https://www.wsj.com/articles/SB115689722809749013) that inspired the 2010 movie “Extraordinary Measures” starring Brendan Fraser as Crowley, Keri Russell as his wife, Aileen Crowley, and Harrison Ford as a researcher Crowley worked with.

Science behind treating and curing diseases is more promising than ever, Crowley said. But political problems, regulatory requirements, instances of exploitative pricing in biotech and middlemen adding further costs are getting in the way of more medical advances.

“We need to get back the magic of biotechnology,” he said.

A slump in biotech stock prices and planned [changes in what the government pays](https://www.wsj.com/health/pharma/uncle-sam-wants-youto-fight-high-drug-prices-42b3edee) for some drugs have hurt companies’ ability to raise money for research and development. Crowley said his mission will be to “prevent bad laws and promote good laws and policies.”

He said he would advocate for patients to get drugs they need and for lower out-of-pocket costs and other policies to ensure universal access and make drugs affordable.

“We’ve got to put patients at the center,” he said.

He will push for less bureaucratic and more flexible processes for approving drugs. And he is pressing for technical [fixes to drug-price controls](https://www.wsj.com/health/pharma/expensive-drugs-from-pfizer-other-companies-targeted-for-first-u-s-price-negotiations-9942b20b) included in the Biden administration’s Inflation Reduction Act. Pharmaceutical companies say parts of the law [discourage investment](https://www.wsj.com/articles/inflation-reduction-drug-prices-11673628922) in the types of drug that often come in pill form and make up about 90% of pharmaceuticals.

While drugs for rare diseases are exempt from price negotiation under the act, the exemption is removed if a drug is approved for more than one indication. Companies often try to get more than one indication for a rare-disease drug to find a wider market and reach more patients.

He also wants to distance the industry from companies that [have given biotech a bad name](https://www.wsj.com/articles/martin-shkreli-banned-for-life-from-drug-industry-ordered-to-pay-nearly-65-million-11642192076) by charging [exorbitant prices for drugs](https://www.wsj.com/articles/martin-shkreli-found-guilty-in-securities-fraud-trial-1501873444) that they didn’t invest in developing, he said.

“We need to call out the bad actors,” he said. 

Crowley built two biotech companies nearly from scratch, fighting his way through scientific setbacks, regulatory challenges and skepticism from investors. He had to balance the needs of his children and other patients and the interests of investors.

“I had to suffer my way to some measure of wisdom, and hopefully I can impart that,” he said.

Amicus, where Crowley was CEO from 2005-2022, has a chief patient advocate, prices drugs at or below similar approved products and urges payers to ensure patients can get them. It also limits annual price increases on its drugs and provides them to patients in its clinical trials for life, whether or not it gets paid, Crowley said.

Crowley makes the industry’s casefor reaping profits—though not outlandish ones—for drugs it develops. Companies invest billions in drugs that don’t work out, he said. Biotech companies should also make it easier for generic manufacturers to make versions of their drugs once patent protections have ended, he said.

“Nobody should go one day without the medicine they need because they simply can’t pay,” Crowley told attendees of the recent conference in Washington. He asked them to “be our conscience” and let BIO know about companies not acting in the interests of patients.

Crowley said a priority for him will be helping the FDA make drug reviews smoother and faster. “They need to think about every drug and every disease individually, and pull out all the tools they have,” he said of the agency, which he said needs more resources.

For life-threatening diseases with no approved drug, regulators should use creative trial designs that avoid placebo groups and make use of real-world evidence of safety and effectiveness, he said. He sees valuable lessons in the race to design and approve [Covid-19 vaccines](https://www.wsj.com/articles/how-hiv-research-laid-the-foundation-for-covid-vaccines-11608821508).

“They didn’t lower the bar and they made sure the vaccines were safe and effective. But they did things [in very creative ways](https://www.wsj.com/articles/moderna-and-pfizer-are-reinventing-vaccines-starting-with-covid-11605638892),” he said.

An FDA spokesperson said that “drugs must undergo a rigorous evaluation of safety, quality and effectiveness before they can be approved.”

The agency also recognizes the differences in the way different drugs are developed, the spokesperson said. “The FDA does not have a ‘one size fits all’ approach, but in weighing the approvability decision considers characteristics of each application such as the seriousness of the disease targeted, the unmet medical need, as well as the proposed indicated population size,” the spokesperson said.

Having survived childhood, Megan and Patrick are in uncharted territory. Doctors and family members can’t anticipate the health threats they might face, Crowley said.

“We can’t ever get comfortable—not until the day there’s a cure,” he said.

Megan said she is feeling better than ever after her October incident. She said she knew when her dad stepped down as Amicus CEO in August 2022 that he would seek a new challenge.

“My first thought was, ‘No way he can sit still long enough, he’ll be annoying us in no time,’ ” she said. “I knew he was itching for another opportunity, a bigger, more important opportunity.”