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HEALTH

Monday, June 2, 2025 B1 Contact: Tom Corwin, tcorwin@postandcourier.com

MEDICAL ADVANCES

# Fighting ‘the worst disease’

## SC biotech company seeking new treatment for devastating skin disease

PROVIDED

Jacqueline Kopelan and her daughter, Rafaella, 17, at their home in Longmont, Colo. Known as Rafi, she and her family have battled epidermolysis bullosa since birth. Rafi was able to receive a drug being developed by a Mount Pleasant-based company and her mother called the effect “almost like a miracle.”

BY TOM CORWIN
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Inside a nautical-themed office building in Mount Pleasant featuring a giant tide gauge and metal fish sculptures, sit quiet offices where people are working to fight “the worst disease you have never heard of,” as one patient put it.

Even as companies seek to help those patients, their families are now staring down threats to their care from potential Medicaid reductions, one advocate and mother said.

Epidermolysis bullosa is a rare and often devastating skin disorder where patients lack the adhesive proteins that hold the different layers of skin together. Each year, there are approximately 200 babies born with it in the United States, and there are currently about 25,000 patients living with it, according to the advocacy group debra of America.

The disease leaves them with fragile skin that can easily blister or tear. In the more severe cases, they quickly shed skin at the slightest touch, leaving behind painful open wounds across much of their bodies. Many of those patients are bandaged and rebandaged daily, an excruciating process that requires multiple painkillers as the bandages take flesh with them as they are removed. Those wounds also close more slowly due to an impaired healing process.

Robert Ryan and others at Paradigm Therapeutics in Mount Pleasant have been working on a drug that has shown in clinical trials it can greatly speed up the wound healing process. One mother that used it called it a “miracle” drug because of how much it reduced the open wounds on her daughter. Now, the company is preparing to go for approval before the Food and Drug Administration, probably late this year.

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# SC company seeking new treatment for skin disease

**TREATMENT**, from B1

From the moment of her birth, it was obvious that there was something different with Rafaella Kopelan. She had no skin on her right hand and her left foot, said her mother Jacqueline. The hospital staff assured her that, because the girl was two weeks late, she was just "overcooked."

That quickly changed.

"Within minutes, her entire body just blew up with blisters and open wounds," Jacqueline said. The child was snatched up and rushed to the neonatal intensive care unit at the hospital in New York City. Her parents didn't see her until a day later, and the staff already had her on a morphine drip to control the pain from her shredded skin. Luckily for the family, a pediatric dermatologist who was visiting the unit had seen a case of epidermolysis bullosa during her fellowship and told the family that she thought that the girl might have it. A biopsy later confirmed it, but the family soon learned the girl they call Rafi would have few good treatment options.

A stem cell transplant failed after she contracted a virus and the family was forced into a now daily hours-long and painful routine of stripping off and layering on bandages over much of Rafi's body.

"I was born with the worst disease you've never heard of," Rafi, then 8, says in a video in 2016 to help raise money for debra of America, over images of her mother struggling to gently remove the bandages as Rafi cries out.

"I know Mom doesn't want to hurt me," Rafi said, but the pain is like "sharp dog's teeth digging into me."

The plight of the Kopelans and other families is what convinced Ryan of Paradigm Therapeutics to work on a better treatment.

"It's probably one of the most painful and devastating diseases that you'll ever see," said Ryan, who sits on the debra board of directors. Having worked in pharmaceutical development for more than three decades, he formed a company that began work on a new treatment while then based in North Carolina. That company merged with another, ran into production problems, and Ryan recently reassumed development of the drug, now called Zorblisa.

It aims to address one of the fundamental problems with many of these patients, like Rafi: They don't have one open wound, they have them all over the body. Paradigm has developed a cream that can be applied all over the body to address that.

The Food and Drug Administration has approved three specific treatments for the disease, two in 2023 and one earlier this year. Two of them aim to restore a key protein, collagen VII, which is part of the anchoring mechanism holding skin layers together. One comes as a credit-card-sized piece that has to be clinically applied to a wound. Others get applied a wound at a time.

"Unfortunately, it's not a single wound disease," Ryan said. Zorblisa would be the first whole-body treatment, he said.

According to the company, the drug works by speeding up the wound healing process, stimulating base layer formation in the bed of the wound that allows for skin and flesh to fill in on top. It also works to stimulate collagen formation and fights the inflammatory response that can deter healing. In clinical trials, patients who used it for three months saw nearly a third of the wounds on their body close and in another trial saw wounds heal twice as fast compared to placebo. It also helped to deter the scar formation that can come with many of these lesions, Ryan said. Importantly, it's proven to be safe for long-term use, which is crucial when treating children, he said.

"We can start treating basically at birth," Ryan said, once approved.

Rafi had a chance to get the drug through compassionate use access early on in the testing. And the effect was evident almost right away, Jacqueline said. She had a chance to get away for a girls' weekend but was checking in on FaceTime with her husband and daughter. They had been applying the drug just to her arms. Rafi appeared on the screen in a sleeveless dress.

"There were no bandages on her arms for the first time ever," Jacqueline said. "It was very exciting. It was almost like a miracle."

PROVIDED

Rafaella "Rafi" Kopelan, 17, of Longmont, Colo., has battled epidermolysis bullosa since birth. She was able to receive for a while a drug being developed by a Mount Pleasant-based company that will seek approval from the Food and Drug Administration later this year.

But access to the drug ended when that clinical trial ended, which was "devastating," she said. Now that the drug could soon be up for approval, "we're so super excited," Jacqueline said. "We cannot wait."

Bad news could be on the horizon for the family if potential cuts to the Medicaid program come about, as advocates fear. The nonpartisan Congressional Budget Office projects that a massive bill that just passed the U.S. House of Representatives could result in at least $625 billion in Medicaid cuts over the next 10 years. While states that have not expanded Medicaid, such as South Carolina, will not fare as badly, other states that did improve services will.

The Kopelan family specifically moved to Colorado because of the expertise in treating Rafi's disease at Children's Hospital Colorado and because it was one of the few states with a Medicaid program that would pay for her bandages, which are specialized and very expensive. It costs $80,000 a month to keep Rafi covered, Jacqueline said. She is also on 18 daily medications, including seven just for pain. Colorado Medicaid is a great program for her, covering everything from her medical supplies to her wheelchair to assistance she needs. Almost all of the patient families like Rafi's are on Medicaid, Jacqueline said, because the care is "phenomenally expensive."

Paying for it on their own would be "impossible," she said.

Colorado is one of the states that would take a big hit from that proposed Medicaid reduction, losing 20 percent of its funding and resulting in 14 percent of its Medicaid population potentially losing coverage, according to an analysis from KFF.

Ultimately, that could affect companies like Paradigm, Jacqueline said. If no one can afford to buy the new drug, is there any incentive for companies to continue developing it, she asked.

"It's very scary with what's happening with Medicaid right now, and we don't know where that's going," Jacqueline said.

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