Saving human lives: Using gene therapy and fetal surgery to cure diseases in the womb

If UCSF is known for birthing the field of fetal surgery, UC Berkeley, located a short drive across the Bay Bridge, is famous in biomedical circles for pioneering CRISPR gene editing, the most powerful DNA-manipulating tool ever invented. What [Pediatric and fetal surgeon Tippi] MacKenzie envisions — the future she is now preparing for — is the convergence of these technologies into a whole new field of medicine dedicated to curing diseases before birth: fetal genome surgery. This is surgery without scalpels or sutures, just a syringe pushing particles containing CRISPR into the vein that connects a pregnant person to the fetus. Once inside the fetus’ cells, CRISPR’s molecular scissors snip away the string of problematic DNA, stopping a catastrophic genetic disease before it really starts. If successful, it would fundamentally and forever change the practice of fetal and maternal care.

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This tantalizing prospect recently led the National Institutes of Health to begin funding projects that are carving a path to clinical trials. Getting permission from the Food and Drug Administration to test a fetal therapy is an enormous challenge, even more so when it involves an emergent technology like CRISPR. Yet, if anyone can do it, scientists told STAT, it’s MacKenzie.

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