How gene therapy could eventually snip out diseases in the womb

In a future when gene therapy can tweak a person’s genome precisely enough to cure them of severe disease, treating earlier will often be better—and the womb is as early as it gets.

Doctors can already detect abnormalities in the DNA of a developing human fetus. Conditions like sickle cell anemia and spinal muscular atrophy arise with genetic signatures—sometimes as simple as a single gene mutation—that appear in prenatal screens. New gene therapies can treat adults and even kids with these conditions, but they have some drawbacks: They can cost millions of dollars for a one-time dose, and many are currently only available to clinical trial participants. Most of all, by the time a person receives them, they may have already spent months, if not many years, living with a serious illness.

Physicians and scientists hope that by correcting these abnormalities before birth, a newborn will stand a better chance of a healthy life. “The main advantage of administering these therapies in the womb or before birth would be to prevent disease before it happens,” says Bill Peranteau, a pediatric and fetal surgeon with the Children’s Hospital of Philadelphia.

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