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GENE THERAPY

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Pharmaceuticals cannot always fix a malfunctioning human body. Sometimes the only way to treat what ails a person is to tinker with their genes: the blueprints for how biological systems are built and how they operate. Some researchers are using gene-editing techniques such as CRISPR to precisely alter DNA sequences. Others are genetically modifying immune cells to imbue them with the ability to fight cancer. And in the past couple of years, there has been a rapid acceleration in the development of a wide range of treatments in which disease-causing genes are replaced in their entirety.

This Outlook therefore focuses on the rich assortment of research in which new genes are introduced into a person, usually by means of a viral vector (see page S18). Successful animal experiments indicate that human genetic disorders could one day be repaired in the womb, so that a baby might enter the world disease-free (S6). And a number of health issues that have proved difficult or impossible to remedy — such as sickle-cell disease (S12), epilepsy (S10) and certain intractable skin conditions (S14) — might be excellent targets for gene therapy.

But gene therapy need not be limited to diseases that originate from genetic abnormalities. It might be possible to treat some viral infections with DNA, by using it to prompt the body into creating just the right monoclonal antibodies to ward off invading pathogens (S16).

Gene therapy remains an expensive medical path, however. Moving it out of the laboratory and into the clinic will require innovative pricing schemes (S23) and regulatory policies (S20). Along the way, clinicians, patients and policymakers will grapple with tricky ethical questions (S9).

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Herb Brody
Chief supplements editor

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