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Carlos Heras-Palou was a 39-year-old surgeon with two young daughters when he developed hereditary transthyretin (hATTR) amyloidosis, a rare degenerative disease caused by the misfolding of a protein called transthyretin (TTR). That diagnosis in 2004 carried a grim prognosis: a typical life expectancy of 3 to 15 years. At the time, most people who developed hATTR amyloidosis needed to be treated with transplant surgery. But another option emerged. Heras-Palou and his younger sister Isabel, who was diagnosed with the condition in 2013, enrolled in studies for a drug that thwarts production of the toxic protein by binding to messenger RNA — the molecule that carries genetic instructions for producing TTR.

Approval of this drug and one other by US and European regulators in 2018 (see page S7) has given fresh impetus to the nascent research field after more than a decade of false starts. That development has raised hopes that RNA therapies in their various forms (S2) can treat not only rare conditions (S16) but also more common ones, including treatments for cardiovascular disease (S13) and vaccines for cancer (S10).

More than a dozen RNA therapies are currently being tested in clinical trials. But major challenges remain, most notably in how to deliver therapeutic strands of RNA into the right cells (S8). Progress will depend on a continuing commitment from pharmaceutical companies to drive and fund innovation (S4). Researchers also need to up their game when it comes to communicating the underlying science of gene manipulation in all its complexity to patients and the wider public (S15).

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David Payne
Managing Editor, Nature Careers and Supplements

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