nature OUTLOOK

HUNTINGTON'S DISEASE

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Cover art: Neil Webb

There are many things that parents would love to pass down to their children. Houses, jewellery, money — all can be welcome gifts from one generation to the next. But not everything that can be bequeathed is so desirable. Huntington's disease — a neurodegenerative condition that causes uncontrollable movements, emotional disturbance and the loss of mental abilities — is an especially unfortunate genetic hand-me-down.

At a glance, the biology of Huntington's disease seems to be simple (see page S36). The condition has been traced to a mutation in a single gene on chromosome 4 that is responsible for producing a protein called huntingtin. And yet fundamental aspects of the molecular and cellular processes that underlie Huntington's disease remain a mystery. Glaringly, researchers have still not worked out huntingtin's role in the cell.

There is no cure on the horizon. But a clinical trial of a potential treatment has raised hopes that such research is starting to bear fruit. The innovative treatment comprises an antisense oligonucleotide — a molecule that binds to messenger RNA to prevent the production of a specific protein (S39). Another possibility is to edit the faulty gene directly (S42). And as researchers pursue these new approaches, they must also wrestle with how to measure success without the need to track the health of patients for years, or even decades, to come (S46).

Huntington's disease is unusual in that its diagnosis generally occurs well into the child-bearing years, which can lead to anguished decisions over whether to take a high-stakes genetic gamble with future offspring (S38). But in rare instances, the disease also strikes children (S44).

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Herb Brody
Chief supplements editor

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