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► **COVER:** 'Orphan drugs' by Susanne Harris, inspired by the Perspective on p519.



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The 1983 US Orphan Drug Act was designed to promote the development of new therapies for rare diseases in the United States. In their Perspective, Coté and colleagues analyse the characteristics and distribution of 25 years of orphan designations and approvals by the US Food and Drug Administration to provide a comprehensive overview of the impact of this legislation. The most common therapeutic area for the development of orphan drugs has been oncology, which is also the focus of the second perspective this month. Peták and colleagues discuss efforts to mirror the success of the pioneering drug trastuzumab in patients identified by molecular diagnostics that detect the human epidermal growth factor receptor 2. Other drugs targeting the epidermal growth factor receptor family have not been as successful as initially hoped, and this Perspective highlights the lessons learned from their development, as well as strategies to decrease the risk of failure by more effectively integrating molecular diagnostics into anticancer drug discovery. Biomarkers that could identify patients who are more likely to respond to treatment or that could help assess the effects of therapies are also much needed in the development of disease-modifying drugs for Alzheimer's disease. In their Review, Hampel and collaborators present the views of academic institutions, industry and regulatory organizations on how biomarker development for Alzheimer's disease may be accelerated. Completing the issue, Ellington and colleagues review the therapeutic potential of aptamers — oligonucleotide sequences that can bind target proteins with affinities and specificities rivalling that of antibodies — while Chao and Henry discuss the inhibition of sodium–glucose co-transporters as a novel therapeutic strategy for diabetes.

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