FT SPECIAL REPORT

FT Health

The Future of Rare Diseases

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Niche drugs market looks set to balloon

Genetics Treatments are being tailored to a specific mutation, writes *Andrew Jack*

In just a few years, rare diseases have moved from neglected "orphan" status into the mainstream of drug development, with a growing number of pharmaceutical companies acquiring and investing in life-saving treatments that benefit targeted groups of patients.

Now they are converging with the broader trend of "personalised medicine" that is transforming research, outcomes and the economics of the industry more widely. "We're moving from blockbusters to niche-busters," says Hilary Thomas, chief medical adviser at KPMG.

Many thousands of orphan conditions affecting small numbers of patients with unusual conditions remain poorly researched with few medical options for treatment and poor prognoses. However, a growing number — from Gaucher's disease, which can cause an enlarged spleen and liver as well as skeletal disorders, to cystic fibrosis, which affects mainly the lungs — have potent treatments that were inconceivable until recently.

That is reflected in growing corporate investment. In January, Sanofi paid nearly $12bn for Bioverativ, a US company focused on haemophilia and other rare blood conditions. Shire, which acquired Baxalta for $32bn in 2016 to strengthen its rare disease portfolio, has been subject to fresh speculation that it will itself be bought after a previous thwarted takeover bid by AbbVie.

EvaluatePharma, a research group, estimates total orphan drug sales will rise from $127bn for 2017 to $217bn in 2022, while their share of the revenue from all prescription medicines will increase from 16 per cent to 21 per cent.

Orphan treatments were long viewed as pioneers in personalised medicines, focused on conditions with a single mechanism and transformative,

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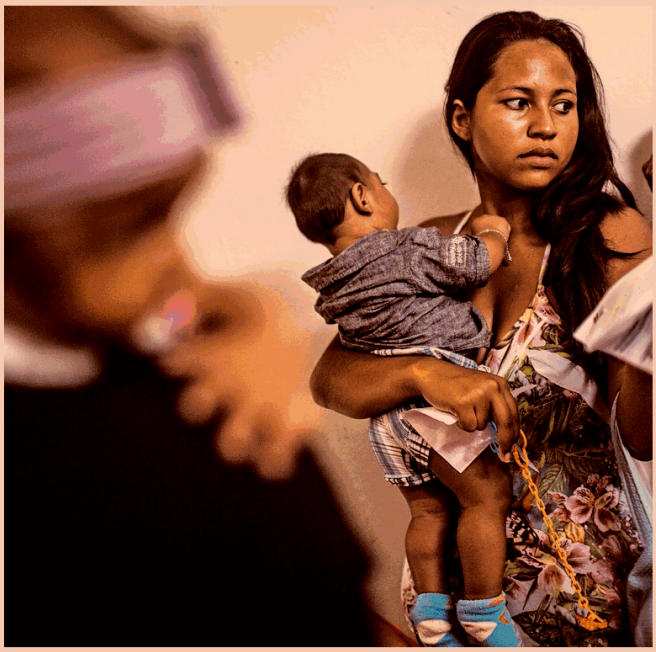
Discovery will aid fight against the horrifying birth defect caused by Zika



Breakthrough: a team led by Brazilian scientist Mayana Zatz has found that only babies with a genetic predisposition are born with microcephaly after infection with the Zika virus. Page 3 — Hilaea Media/Dado Galdieri for Financial Times

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