Evaluate Vantage

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Rare diseases top another strong year for novel drug approvals

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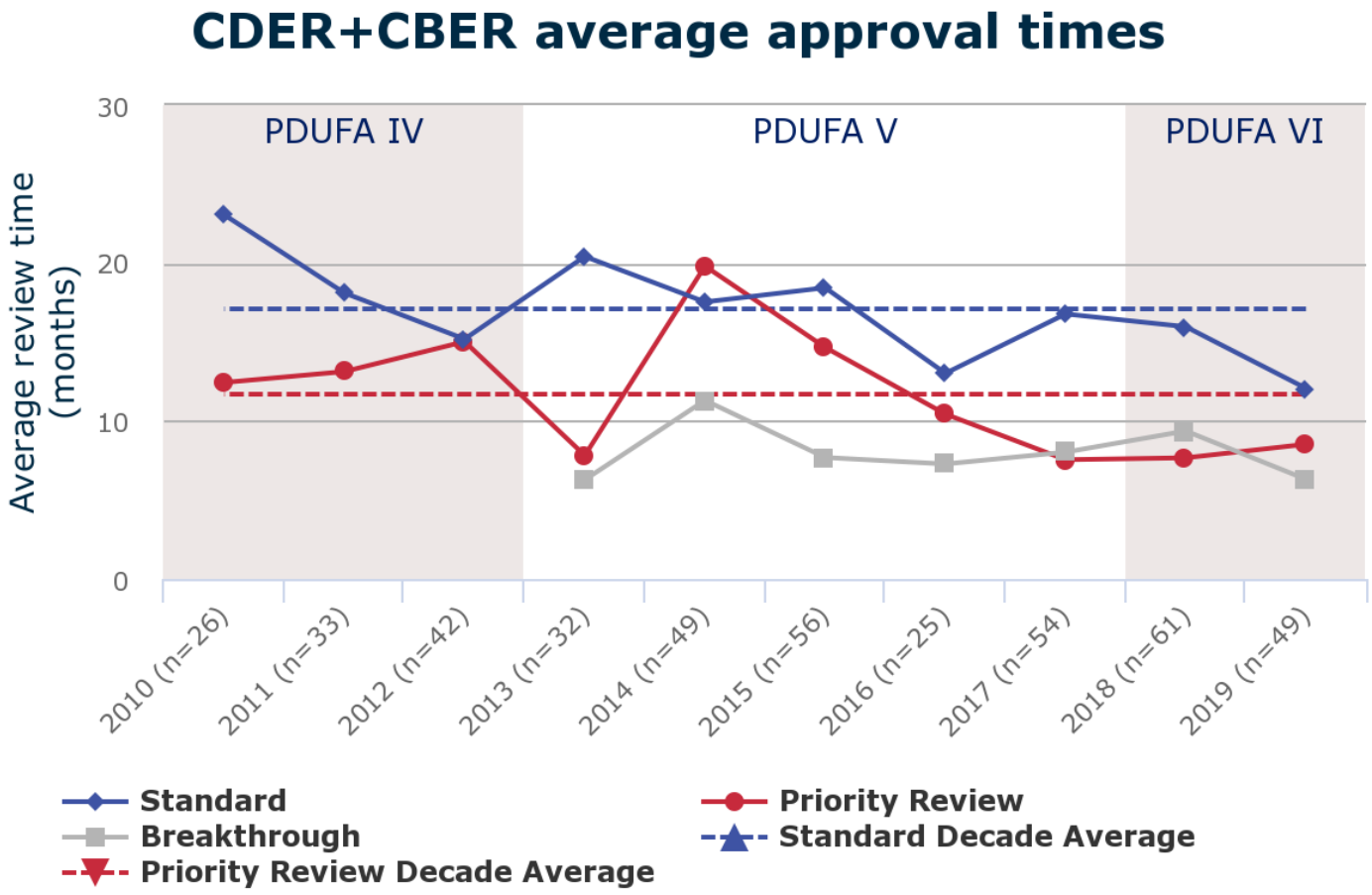
A clutch of swift FDA decisions boosted average US drug approval times in 2019, helping biopharma to deliver another bumper crop of new medicines.

Towards the end of last year several surprisingly speedy FDA approvals helped put the wind in biopharma's sails. A look at the picture across the whole of 2019 confirms that the US drugs regulator remains one of the sector's best friends.

Over the year the agency approved 49 novel medicines with a fifth-year sales potential of $27.1bn, according to a *Vantage* analysis of *EvaluatePharma* data. Neither of these numbers is a record, but they are far from disappointing, and certainly fit within a new regulatory normal that has been developing over the past six years.

What did improve markedly last year was average approval time in the breakthrough designation category, to give the regulator its best score since 2013; the 6.3-month mean is even more remarkable considering that in that year, when BTD was first introduced, only three projects used this pathway. In 2019 the average was derived from the review of 15 projects.



Standard reviews came in bang on 12 months, on both median (not shown here) and mean measures, though this analysis suggests that the FDA struggles to get priority reviews to hit the six-month target it aims to. The past three years has shown some improvement at least, with the average priority review taking around eight months.

Last year a sense of speed certainly emerged from the FDA, though only two of the class of 2019 made it into the 10 fastest approval decisions since 2010. Behind Trikafta and Enhertu there were plenty of other quick reviews last year, however: 11 drugs were approved in less than six months, almost a quarter of all of 2019's submissions.

Vertex’s new cystic fibrosis triplet, Trikafta, stands out here. Not only was it approved the fastest last year, it is also projected to become the most commercially successful. The sellside’s consensus sits at $3.9bn in US sales by 2024; given the extensive revenues that the company’s existing suite of cystic fibrosis drugs bring in, there will be a fair amount of certainty attached to this figure.

Astrazeneca and Daiichi Sankyo’s Enhertu also scraped into the top 10, with accelerated approval in late-line breast cancer. US sales of $1.4bn are estimated for 2024, but the real commercial future of this antibody-drug conjugate is still hard to determine; a big unknown is how its toxicity profile will influence its use, and perhaps chances of approval in earlier lines of therapy.

Green light ahead: the fastest FDA decisions since 2010			
Product	**Year approved**	**Status**	**Months to approval**
Blincyto	2014	Breakthrough therapy	2.5
Iclusig	2012	Priority review	2.6
Jevtana	2010	Priority review	2.6
Spinraza	2016	Priority review	3
Alecensa	2015	Priority review	3.1
Trikafta	2019	Breakthrough therapy	3.1
Xtandi	2012	Priority review	3.3
Kalydeco	2012	Priority review	3.5
Zelboraf	2011	Priority review	3.6
Enhertu	2019	Breakthrough therapy	3.7
Source: EvaluatePharma.			

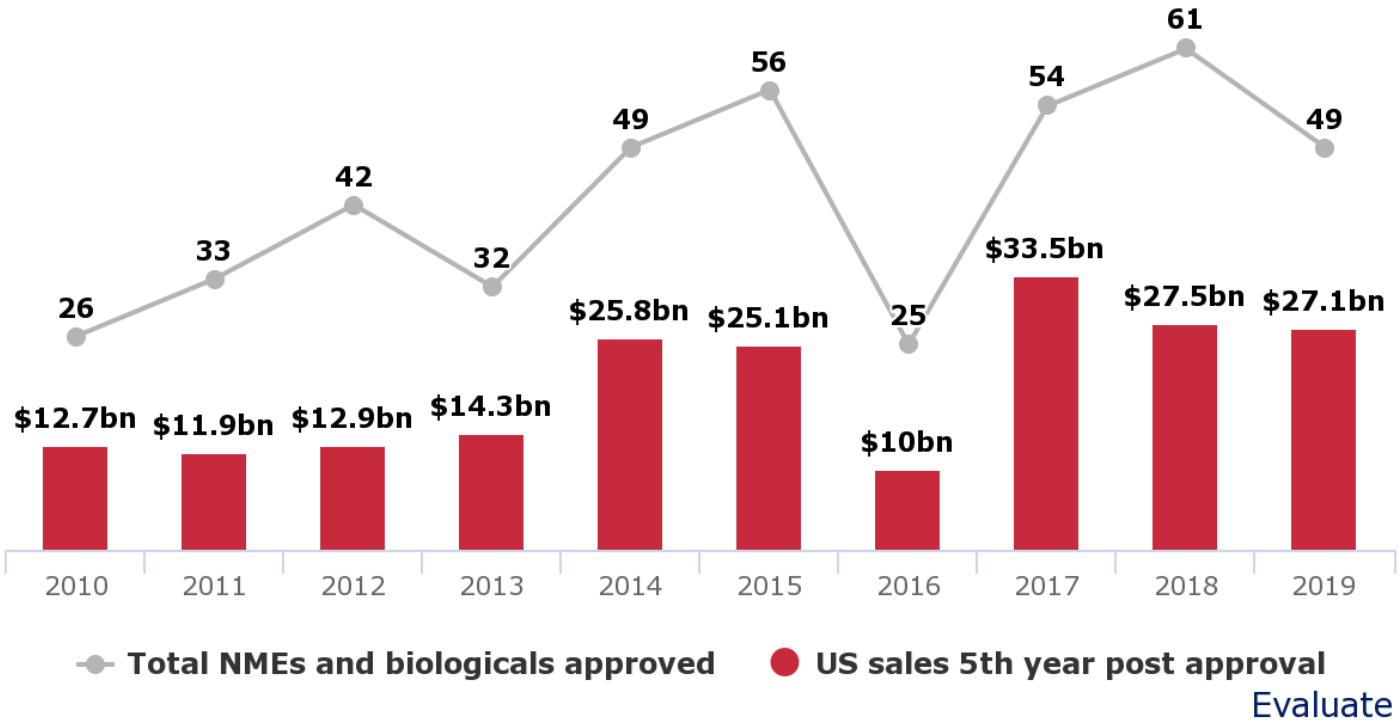
The chart below looks at the total commercial potential of last year's approvals; the $27.1bn figure was boosted by the arrival of nine future blockbusters, four of which are predicted to be bringing in more $2bn in the US by 2024. As well as Trikafta, this includes two new arrivals for Abbvie: the psoriasis antibody Skyrizi and the Jak inhibitor Rinvoq for RA.

Pfizer's amyloidosis treatment Vyndaqel is also seen as having a hugely successful future, with 2024 sales of $2.5bn pencilled in. This therapy and Trikafta both treat rare diseases and are prime examples of the sort of products that biopharma is chasing right now: small, definable patient populations in which big price tags are accepted.

While the FDA is also happy to approve these rare disease drugs very quickly, there are few reasons to believe that biopharma will turn away from this space anytime soon. What looks to be internal disagreement at the agency around the approval of Sarepta's Duchenne muscular dystrophy agents perhaps raises a red flag, but there is little evidence of internecine warfare more broadly (*Internal FDA documents reveal a familiar story for Sarepta, January 22, 2020*).

A big safety scare can quickly change everything, of course, but as things stand few are expecting substantial in the regulatory landscape this year.

FDA approval count vs. 5th year US sales



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