Evaluate Vantage

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Flurry of FDA activity raises hopes for 2017 approval tally

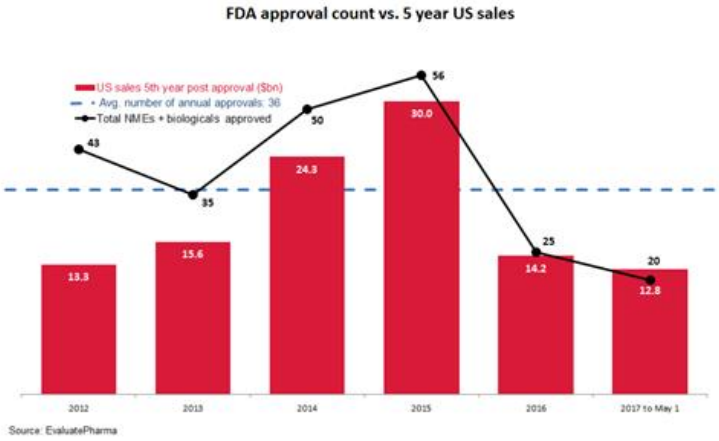
Amy Brown

Five green lights from the FDA over the last few days will be read as an encouraging flurry of activity for those concerned about the pace of US approvals. The busy spell means this year is highly likely to beat 2017 on the number of novel drug approvals (see table below).

Of the recent clutch, Astrazeneca's checkpoint inhibitor Imfinzi is expected to become the biggest commercial success; it ranks as the third biggest approval so far this year, according to *EvaluatePharma*. Novel agents from Novartis and Biomarin, meanwhile, are notable for being the first drugs to reach the market to treat rare forms of leukaemia and Batten disease.

These five additions make 20 novel drug approvals so far this year in the US – as well as the 19 from CDER, the agency's biologics division CBER granted approval to Alk-Abelló's dust mite allergy vaccine.

The graph below also confirms that the value of 2017's new drugs – based on fifth year US sales forecasts – is on track for uplift. Imfinzi is perhaps a big swing factor here – the $2.3bn figure that the sell-side has pencilled in for 2022 is highly dependent on trials in lung cancer reading out successfully in the coming months. Its initial approval in bladder cancer, a crowded space, will account for a small proportion of those sales, if all goes well (*Therapy focus – Immunotherapy crowds bladder cancer space, May 2, 2017*).



Biomarin's Brineura was designed to treat children with CLN2 disease, a very rare progressive fatal brain condition that typically restricts life expectancy to 12 years of age. The enzyme replacement therapy is administered directly to the brain, replacing the TPP1 enzyme (*Biomarin and the $702,000 question, April 28, 2017*).

Rydapt meanwhile has been approved in two indications – FLT3-mutated acute myeloid leukaemia and advanced systemic mastocytosis – and represents the first targeted agent to show efficacy and win approval in either indication.

The drug is a multi-targeted kinase inhibitor that can hit the main drivers of these diseases. A third of AML patients carry a FLT3 gene mutation while most cases of systemic mastocytosis are caused by a KIT gene mutation.

Also last week Radius Health won approval for its osteoporosis candidate Tymlos, a parathyroid hormone analogue that will now seek to carve a space in a competitive market. The product was approved with a black box warning of the risk of osteosarcoma and the regulator did not allow any superiority claim over other agents. Though this was broadly expected the final label dashed lingering hopes of greater appeal.

The news also probably further limits the chances of a takeout, rumours of which have lifted the stock at various points over the last couple of years. The company is now worth $1.5bn, half its 2015 peak. With Amgen's new osteoporosis agent Evenity due to hear on FDA approval by July 19, Radius will have to execute a very strong launch to keep investors interested.

Finally Takeda received partial validation of its $5.2bn takeout of Ariad with approval of Alunbrig, to treat patients with ALK positive NSCLC who have progressed on Pfizer's Xalkori. The drug is the fourth in its class to reach the

market, and the Japanese company will have to work hard to carve a niche (*Therapy focus – Roche looks ready to rule another cancer niche, 6 March 2017*).

And the rest?

There have also been notable disappointments as well this year – most importantly the delay to baricitinib, Eli Lilly and Incyte's Jak inhibitor which was turned down in RA last month.

With Ocrevus and Dupixent already approved, the big decisions of the year are already in the bag. Verdicts on Novartis and Kite Pharma's CAR-T projects remain the closely watched regulatory events on the horizon for oncology; in diabetes Novo Nordisk will be hoping for good news on its once-weekly GLP-1 agonist before the year is out.

Unless approvals grind to a halt fears of a decline in productivity should prove misplaced. However for a return towards the 10 year average the pace must be maintained.

To contact the writer of this story email Amy Brown in London at AmyB@epvantage.com or follow @ByAmyBrown on Twitter.