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# Pharma regulatory news over the Christmas period

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Christmas, a time when investors are likely to be focused on jollity and taking a well-earned break from the year's hard work, presents an ideal opportunity for companies to bury bad news. But not this time.

On the contrary, the past week or so has been characterised by regulatory approvals, with only a couple of setbacks reported. In addition to Eliquis, approved three months before the FDA's action date and just six months after being knocked back by the agency, the US regulator has nodded through five drugs (*Bristol-Myers and Pfizer ring in 2013 with Eliquis approval, January 2, 2013*). And in a year-end flurry of activity the FDA's opposite number in Japan has also been busy.

*December 31*

As the champagne was being chilled on New Year's Eve, the FDA gave Salix Pharmaceuticals and Johnson & Johnson additional reasons to toast 2013, announcing approvals for crofelemer and bedaquiline.

Salix's crofelemer, a CFTR channel & calcium-activated chloride channel blocker trade-named Fulyzaq, is the first anti-diarrhoeal drug to get the green light for use specifically in HIV/AIDS patients, as well as becoming only the second botanical, after Medigene's Veregen, to gain US approval. Shares in Salix crept up, as did those in Glenmark Pharmaceuticals, which manufactures the crofelemer API.

The regulatory go-ahead for J&J's bedaquiline (Sirturo) came a month after the drug received unanimous advisory committee backing for use in multidrug-resistant tuberculosis *(Event – J&J drug for resistant TB speeds towards US approval, November 29, 2012).* Strangely, though, the FDA kept its positive decision under wraps until three days after the December 28 action date on which it had been taken.

Earlier, the US consumer advocacy group Public Citizen had urged the FDA to reject bedaquiline, citing an increased risk of death for patients on the drug. Instead, the agency slapped a boxed warning on Sirturo's label relating to QT prolongation, as well as patient deaths.

*December 25*

On Christmas Day Novo Nordisk scored a small victory in securing Japanese approval for Ryzodeg, its combination of the long-acting insulin degludec with the fast-acting insulin aspart; the long-acting ingredient, Tresiba, got the green light in Japan in September. The real prize, of course, remains the US FDA's verdict on both Tresiba and Ryzodeg. They scraped through a surprise adcom two months ago (*Doubts linger after Novo Nordisk insulins scrape through US adcom, November 9, 2012*).

The Japanese Ministry of Health also gave UCB a double reason to celebrate, nodding through Cimzia for adult rheumatoid arthritis and Neupro for Parkinson's disease and restless legs syndrome. The two approvals were not unexpected but capped what analysts at Bryan, Garnier & Co called a very great year for UCB, whose stock ended 2012 up 36%.

*December 24*

A day earlier the US FDA approved Aegerion Pharmaceuticals' lomitapide as Juxtapid – its broad label hardly surprising given the drug's strong advisory panel endorsement for treating the tiny indication of hereditary familial hypercholesterolaemia.

An EU decision is still awaited and is perhaps less certain after the CHMP issued a negative opinion of Isis Pharmaceuticals' Kynamro, although this competitor has also received a far more cautious US panel backing than lomitapide did (*Panel votes presage fight for tiny hypercholesterolaemia population, October 19, 2012*).

*December 21*

NPS Pharmaceuticals' short bowel syndrome drug Gattex had secured a positive adcom vote last year, and its troubled path to US approval ended happily after all when the FDA blessed it just as the holidays were getting

under way.

Similarly, development of Alexza Pharmaceuticals' Adasuve for agitation associated with schizophrenia or bipolar disorder had been anything but plain sailing, but the drug, which the FDA declined to approve in May, got the green light on December 21. Still, the label is restrictive, and the tiny Alexza has yet to find a US commercial partner.

Meanwhile, Raptor Pharmaceutical found itself out of luck when the FDA delayed by three months the PDUFA date for its nephropathic cystinosis project Procysbi. A little earlier the company had mortgaged the asset to HealthCare Royalty Partners to raise a much-needed $50m in royalty financing, and its shares have already recovered from the resulting dip, showing the degree of confidence in Procysbi's approval by the new April 13, 2013 action date.

Importantly, the US agency has not demanded new studies of Procysbi, and so the delay stands as a relatively minor black mark on a Christmas break full of festive cheer on the regulatory front.

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