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Pharma data news over the Christmas period

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While the regulatory news managed to surprise investors, what with the earlier than expected approval of Eliquis and a big finish for the end of the year with respect to approvals, it was a little quieter on the data front this Christmas, as only seven companies announced news of note from the labs (*Bristol-Myers and Pfizer ring in 2013 with Eliquis approval, Jan 2, 2013*).

Of those companies, Biotie, Transcept and Isotechnika Pharma had obviously managed to get themselves on the naughty list and missed out on the present of positive data, sending their shares down. Conversely, those with something to celebrate included Actelion and Pergamum, which both achieved success in the difficult anti-infective space, and Spectrum Pharmaceuticals, which has one of the few histone deacetylase (HDAC) inhibitors in development, reporting positive phase II results in relapsed/refractory peripheral T-cell lymphoma (PTCL).

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Isotechnika might have been hoping that 2013 would be the start of some positive news for its eye drug voclosporin, following several development disappointments over the last two years including a complete response letter for the drug in non-infectious uveitis in August 2010. That setback saw the FDA request an additional trial before it would reconsider the drug for approval.

In what was the coup de grace for the drug, that additional phase III trial, conducted under license by Lux Biosciences, failed to meet its primary endpoint of clearing the haze associated with the inflammation of the middle layer of the eye. The company is not expected to continue with a filing of the drug either in the US – again – or in Europe. All, however, may not be lost as the drug has been filed in psoriasis and is in phase III development for kidney transplantation, and had only been licensed to Lux in ophthalmology in the hope of generating a new revenue stream.

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Isotechnika may not have been depending on a win to change its fortunes, but the same cannot be said for Omeros, which managed to produce mixed data for its knee treatment drug OMS103HP. While hopes have not been high for the drug thanks to its previous patchy development record, it is still expected to be the group's second highest royalty revenue earner, according to forecasts from *EvaluatePharma* (*Event – Omeros hopes for double success for surgical pain relief candidates, October 8, 2012*).

In a 344 patient phase III trial, OMS103HP failed to meet the primary endpoint of improving the performance of knee joints after surgery, reversing the findings of a phase II trial. There was some comfort for weary investors after the drug hit its secondary endpoint of significantly reducing postoperative pain.

Indeed, the results in pain reduction were strong enough for Omeros to vow to plough on with a second phase III trial, which is due to start in the second half of 2013. The big question hanging over the drug will be whether any partners will be willing to get on board at this point. If not, the group, which has about $31m in the bank, might have to look to investors to fund any further development.

Developing products in CNS has always been a tough nut to crack, and Biotie's dopamine beta hydroxylase inhibitor nepicastat (SYN-117) was added to the list of failed products after it failed to alleviate symptoms of post-traumatic stress disorder in combat veterans in a US Department of Defense sponsored trial. But the drug could have a second bite at the cherry as Biotie is set to continue to develop nepicastat in cocaine dependence, again using US government money, working this time with the National Institute on Drug Abuse. Patient enrollment is expected to start in the first quarter of 2013.

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Transcept also saw its hopes dashed over another CNS product, as ondansetron (TO-2061) proved to be no better than placebo as a second-line treatment for obsessive compulsive disorder. Shares in the company fell 6% on the news to $4.85. With little else in the pipeline, and the company's only other filed drug being the

poorly performing sleeping pill Intermezzo, 2013 could be an uncomfortable year for Transcept.

There was much better news on the anti-infective front for both Pergamum and Actelion. Private Swedish biotech Pergamum is now on the hunt for a development partner after DPK-060, a treatment for acute external otitis or swimmer's ear, showed positive results in a small 69-patient phase II trial. Between 85-94% of patients showed a complete cure after 10 days of treatment. What might get partners interested in the drug is that its novel composition, as an antimicrobial peptide derived from the human protein kininogen, could avoid the problem of antibiotic resistance.

Actelion also has plans to advance its drug cadazolid to phase III following a good showing in an 84-patient phase II dose finding-study in *Clostridium difficile*-associated diarrhoea (CDAD). The trial pitted the drug against vancomycin, but only as an active reference. But the comparison did little to harm cadazolid, which at least matched vancomycin's performance at all doses, exceeding it at some. At present, peak sales for the product are only expected to reach $509m; while not a blockbuster it could be a welcome addition to revenues if it makes it past phase III, the stumbling block for many anti-infectives.

There was also good news for novel drugs, as Spectrum Pharmaceuticals did its bit to advance the cause of HDAC inhibitors, after the phase II Belief trial of belinostat in relapsed/refractory peripheral T-cell lymphoma achieved an objective response rate of at least 20%. Spectrum now intends to file the drug by the middle of this year, meaning approval could come at some point in 2014. If it is approved the drug could potentially be the fourth HDAC on the market, coming just behind Novartis' panobinostat. Shares in Spectrum rose by 4% to $11.72.

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