BIOBUSINESS BRIEFS

MARKET WATCH

Upcoming market catalysts in Q2 2017

Important market catalysts expected in the second quarter of 2017 include regulatory decisions by the FDA on cerliponase alfa (developed by BioMarin) for treatment of CLN2 disease and brigatinib (developed by Takeda) for the treatment of non-small-cell lung cancer (NSCLC), as well as top-line phase III data for the combination of netarsudil and latanoprost (developed by Aerie Pharmaceuticals) for the treatment of glaucoma or ocular hypertension.

Cerliponase alfa is a recombinant form of the enzyme tripeptidyl peptidase 1 (TPP1). It is intended to treat children with CLN2 disease, a rare inherited lysosomal storage disorder caused by deficient TPP1 activity, which primarily affects the nervous system. During the initial review of the biologics licence application (BLA) for cerliponase alfa, the FDA requested an updated efficacy data cut from an ongoing extension of the pivotal study submitted in the BLA, which the company provided. The FDA designated the submission of the additional data as a major amendment to the BLA, resulting in an extension of the initial Prescription Drug User Fee Act (PDUFA) action date by 3 months, to 27 April 2017. An FDA advisory committee meeting is expected leading up to the PDUFA date. Efficacy appeared encouraging, but a major topic of discussion is likely to be the adequacy of the natural history comparator, and the related benefit to risk ratio in light of the drug-related serious adverse events. If approved, cerliponase alfa would be the first approved drug for CLN2 disease.

With the recent acquisition of Ariad Pharmaceuticals earlier this year, Takeda gained brigatinib, an anaplastic lymphoma kinase (ALK) inhibitor. Takeda is seeking approval for brigatinib for treatment in patients with metastatic ALK-positive (ALK^+) NSCLC whose disease has progressed while receiving the ALK inhibitor crizotinib (Xalkori; Pfizer). The FDA granted breakthrough designation to brigatinib in October 2014 based on results from a phase I/II trial that showed sustained antitumour activity in ALK^+ NSCLC patients. The new drug application (NDA) for brigatinib is under priority review, with a PDUFA action date of 29 April 2017. The approval of brigatinib could add to the existing subset of NSCLC drugs for patients with ALK^+ disease, which includes the pioneering drug crizotinib, as well as the second-generation drugs ceritinib (Zykadia; Novartis) and alectinib (Alecensa; Roche), both of which have activity in patients whose disease has progressed while receiving crizotinib.

Netarsudil, a novel Rho kinase and noradrenaline transporter inhibitor that targets the trabecular meshwork, is currently being reviewed as a monotherapy for the treatment of glaucoma and ocular hypertension. Following the recent NDA resubmission on 28 February 2017, Aerie expects a standard 12-month FDA review process for the monotherapy, with a product launch anticipated in the second quarter of 2018. Aerie is also developing a once-daily eye drop that combines netarsudil with latanoprost, a prostaglandin analogue that is widely prescribed for glaucoma. If approved, this combination would be the first glaucoma product to lower intraocular pressure through four different mechanisms: increasing fluid outflow through the trabecular meshwork (the eye's primary drain); increasing fluid outflow through the uveoscleral pathway (the eye's secondary drain); reducing fluid production in the eye; and reducing episcleral venous pressure. The potential approval will be based on two pivotal trials. The first study (MERCURY I) showed that the combination product is statistically superior to each of its components in lowering intraocular pressure. A second identically designed pivotal trial (MERCURY II) is expected to report results in the second quarter of 2017. Pending positive results, Aerie plans to submit an NDA in late 2017 or early 2018.

Eric Ho is at Informa, 3655 Nobel Drive, San Diego, California 92122, USA.
Eric.Ho@informausa.com

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