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MARKET WATCH

Upcoming market catalysts in Q1 2014

Notable market catalysts for the first quarter of 2014 include a US Food and Drug Administration (FDA) advisory panel meeting for fluocinolone acetonide (Iluvien; Alimera Sciences/pSivida) for diabetic macular oedema, and an FDA approval decision for elosulfase alfa (Vimizim; BioMarin), which is a treatment for mucopolysaccharidosis IV (MPS IV; also known as Morquio A syndrome). Also expected in the first quarter are Phase III data from Gilead's highly anticipated fixed-dose combination (FDC) of sofosbuvir and ledipasvir in treatment-naive patients with genotype 1 hepatitis C virus (HCV) infection.

Alimera originally submitted an application for US approval of the corticosteroid fluocinolone acetonide for diabetic macular oedema — a complication of diabetes that often leads to blindness — in June 2010. The company received a complete response letter from the FDA in November 2011 citing the lack of sufficient data to support approval and requesting that two additional clinical studies be completed. Although the drug was approved in multiple European countries in 2012, it was not until March 2013 that Alimera was able to resubmit an application to the FDA using data from the two previously completed Phase III studies, as opposed to new studies that the FDA had requested. The FDA again issued a complete response letter in October 2013, citing concerns regarding the benefit–risk profile. Along with this second rejection, the agency announced that an advisory panel meeting will take place on 27 January 2014 to assist the companies in addressing the deficiencies and provide advice regarding the patient population. As diabetic macular oedema has become a lucrative market for both ranibizumab (Lucentis; Roche/Novartis) and aflibercept (Eylea; Regeneron/Bayer), we expect the advisory panel's decision to be market-moving.

BioMarin is developing elosulfase alfa as a treatment for MPS IV, a rare inherited metabolic disease that leads to abnormal development in infancy and often early death. The regulatory application was submitted to the FDA in April 2013 and was granted priority review. On 19 November 2013, an FDA advisory committee voted 19 to 1 for approval for all patients with MPS IV, along with 1 vote recommending approval only in a subgroup of patients who demonstrated worse baseline walking abilities as demonstrated by the 6-minute walk test and 3-minute stair climb test. The FDA is expected to make an approval decision on elosulfase alfa by 28 February 2014.

> This fixed-dose combination is also anticipated to be the first interferon-free, single-pill regimen for patients with genotype 1 HCV.

Gilead will release top-line Phase III data for the FDC of sofosbuvir, an HCV NS5B polymerase inhibitor, with ledipasvir, an NS5A replication inhibitor. Gilead is studying this FDC in three Phase III studies, known as the ION studies (ION 1–3), which include treatment-experienced as well as treatment-naive patients with genotype 1 HCV — the most common genotype. At an FDA advisory panel meeting in October 2013, panelists unanimously voted to recommend approval of sofosbuvir in combination with interferon and ribavirin for the treatment of patients with genotype 1 HCV, as well as in combination with ribavirin alone for patients with genotype 2/3 HCV. This FDC is also anticipated to be the first interferon-free, single-pill regimen for patients with genotype 1 HCV. The first results from the 800-patient ION-1 study of treatment-naive patients with genotype 1 HCV receiving treatment for 12 and 24 weeks in combination with ribavirin are expected in the first quarter of 2014. Given the impressive data already seen with this combination, we expect promising results from this later-stage study.

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Competing interests statement
The author declares no competing interests.