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Europe: Cross-Country HTA of Gene Therapy Libmeldy Calls For Price Cut

by Francesca Bruce

Joint price negotiations for Orchard's gene therapy Libmeldy could follow outcome of multi-country health technology assessment.

Orchard's one-time gene therapy Libmeldy (atidarsagene autotemcel) should only be reimbursed if the company offers a significant price reduction, concludes a health technology assessment (HTA) report from BeNeLuxA, a cross-country initiative in Europe focused on access to medicines. Joint pricing and reimbursement negotiations between Orchard and three countries over what is reported to be the world's most expensive drug could now follow.

BeNeLuxA aims to make access to medicines more sustainable through cooperation on HTA, pricing and reimbursement, policy exchange and horizon scanning. It is made up of Belgium, the Netherlands, Luxembourg, Austria and Ireland. Only Ireland, the Netherlands and Belgium joined forces for this assessment of Libmeldy, for treating children with metachromatic leukodystrophy (MLD), which is a rare, life-limiting neurodegenerative disorder. (Also see "*Gene Therapy Libmeldy Undergoes Cross-Country HTA In Europe*" - Pink Sheet, 4 Mar, 2022.)

Ireland's HTA body, the NCPE, conducted the cost-effectiveness component of the joint assessment, with input from the Dutch HTA body, ZIN. Meanwhile, the Belgian HTA body, RIZIV-INAMI, conducted the pharmacotherapeutic and budgetary component.

Joint pricing and reimbursement negotiations could follow the BeNeLuxA HTA report if Orchard and the participating countries agree. Last year, Novartis pursued joint negotiations with the same three countries to secure reimbursement for its spinal muscular atrophy gene therapy, Zolgensma (onasemnogene abeparvovec). The company said it pursued this route to reimbursement as it would mean quicker access across the three countries. (Also see "*Three EU Countries Strike Landmark Joint Zolgensma Pricing Deal*" - Pink Sheet, 20 Oct, 2021.)

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The BeNeLuxA joint assessment for Libmeldy concluded that the benefit of gene therapy compared to best supportive care "appears to be marginally greater" in patients who have not yet developed symptoms and in patients in the late infantile subgroup, said a summary of the findings published by the NCPE. It added that robust data informing classification of response, assumption of cure and duration of response were limited.

The report said that the uncertainty associated with both the added clinical benefit and cost effectiveness of Libmeldy compared to best supportive care was considerable. "For all countries, a significant price reduction would be required to reduce the uncertainty with regard to cost-effectiveness," said the summary. The wholesaler price is €2,875,000 ($2,787,571) per dose.

Regarding Libmeldy's use in Ireland, the five-year cumulative gross drug budget impact was found to be €9.94m. A cumulative three-year budget impact was required for Belgium and the Netherlands, which was found to be €6.1m and €14.38m for these markets respectively. Net budget impacts were found to be similar as there are no appreciable cost offsets from comparator therapies.

The NCPE has recommended that Libmeldy should not be considered for reimbursement unless cost-effectiveness can be improved compared to existing treatment.

Meanwhile, ZIN recommended that Libmeldy should be reimbursed for children with MLD who do not yet show any symptoms. However, it said it should only be reimbursed if the health ministry and Orchard agree a pay for performance, which links payments to how well a product works in real life.

MLD is an extremely rare, rapidly progressing and fatal neurodegenerative disease for which there are no other approved treatments beyond palliative care, a company spokesperson told the Pink Sheet. "Libmeldy was approved by the European Commission for the treatment of MLD in eligible patients based on data encompassing 10+ years of follow-up in the earliest treated patients. Many gene therapies have not or will not come to market with that amount of evidence and follow-up data," he said.

The spokesperson added that the company's ongoing dialogue with Belgian, Dutch and Irish reimbursement authorities would focus on "reinforcing the value proposition of Libmeldy—including the strength of these longer-term data, which demonstrate stable cognitive and motor function—as well as securing reimbursed access for eligible patients."

The company remains "open to flexible payment and outcomes based arrangements," he said.

Orchard's chief commercial officer, Braden Parker, told the *Pink Sheet* in May that the company's

deals were "a little different" in each country. “We are very willing to offer up different types of payment arrangements that work for those individual markets,” he said. (Also see "*Orchard Secures New Libmeldy Price In Germany*" - Pink Sheet, 5 May, 2022.)

Earlier this year, Orchard agreed to a 14% discount on the original list price of Libmeldy in Germany. In February, Orchard also offered a “significant” but confidential discount on the list price for Libmeldy in England. (Also see "*Libmeldy: ‘Significant Discount’ For World’s Most Expensive Drug Secures English Funding*" - Pink Sheet, 4 Feb, 2022.) An agreement has also been inked in Italy. The Orchard spokesperson says that these deals "recognize the value of Libmeldy commensurate with its clinical impact."