20 YEARS

SCRIP AWARDS 2024

WEDNESDAY 4 DECEMBER 2024 | RAFFLES LONDON AT THE OWO

EXAMPLE ENTRY FORM

BEST NEW DRUG

ENTRY DEADLINE: FRIDAY 12 JULY 2024

Best New Drug
Category Criteria

The Best New Drug Award recognises excellence in pharmaceutical development. Launching innovative new products is the most important function of the industry and a successful new drug launch marks the culmination of years of risky and expensive R&D. In this category, the judges will be looking for the small molecule or biological product that was approved in its first market worldwide during the qualifying period that represents the best therapeutic advance in its area.

All recently launched new active substances, including small molecules, biologicals or vaccines, are eligible to enter as long as they were first licensed in any market between 1 June 2022 - 30 June 2023. Generic and biosimilar products are not eligible for this Award.

Entrants must have played a role in the development of the nominated product, and all parties to joint ventures should be disclosed in the application.

Disclaimer: The companies, drugs, diseases and people in these entries are entirely fictional. Any resemblance to actual companies, drugs, diseases and persons living or dead is entirely coincidental.

Grande Pharma Plc's Scrip Award Entry For Best New Drug:
XBEVIA (omelogibob) For Bevial's Disease

250-WORD (MAX) SUMMARY:

Grande Pharma Inc launched its first-in-class therapy XBEVIA (generic name: omelogibob) for the treatment of Bevial's disease in its first market, the US, in October following an approval from the US Food and Drug Administration on 30 September. The FDA approval is based on the BEV-UP trials, the first Phase III clinical trials in the field of Bevial's disease.

XBEVIA was licensed for development and commercialization from CuttingEdge Biotech plc for the treatment of Bevial's disease, a rare disease that affects approximately 100,000 people in the US and 110,000 in Europe. It typically occurs in people over the age of 45 and tends to affect more women than men.

XBEVIA is an oral inhibitor of logibobin which sits at the apex of the logibobin cascade, part of the Bevial system in the lungs. XBEVIA is the first drug to modulate this target and act on the underlying disease process. It is also the first pharmaceutical to show a significant effect on the key symptoms of Bevial's disease in a prospective, randomized Phase III trial. Before the approval of XBEVIA, treatment was confined to off-label inhaled corticosteroids and bronchodilators such as beta- 2 agonists for acute exacerbations.

The estimated market opportunity is $1bn in the US, and a similar amount in the rest of the world. Since XBEVIA's approval (launching 1 October), it generated $20m in net product revenue in its first quarter on the market.

ENTRY (1,500 WORDS):

Please give the trade and generic name of the entrant, the therapeutic indication for which it is being considered, and the company or companies involved in its development (if more than one company, please detail the relationship between the parties)

XBEVIA (generic name: omelogibob) was developed by Grande Pharma Inc (licensed for development and commercialization from CuttingEdge Biotech plc) for the treatment of Bevial's disease.

What was the date and market of the candidate's first approval and launch?

The US Food and Drug Administration (FDA) approved the New Drug Application for XBEVIA to slow the rate of decline in pulmonary function in adults with Bevial's Disease on 30 September 2022. It was launched the following month. To date, XBEVIA has received further marketing authorizations in the EU and UK.

Please explain the mechanism of action of the candidate. How does this represent a step forward in treatment in this therapeutic area?

XBEVIA is an oral inhibitor of logibobin which sits at the apex of the logibobin cascade, part of the Bevial system in the lungs.

Bevial's disease is a rare interstitial lung disease in which the Bevial system is dysregulated to produce a thickening of the alveolar walls. These irreversible scarred lesions result in reduced lung capacity, lower blood oxygenation and respiratory distress.

XBEVIA is the first drug to modulate this target and act on the underlying disease process. It is also the first pharmaceutical to show a significant effect on the key symptoms of Bevial's disease in a prospective, randomized Phase III trial. Before the approval of XBEVIA, treatment was confined to off-label inhaled corticosteroids and bronchodilators such as beta- 2 agonists for acute exacerbations.

XBEVIA received Breakthrough Therapy Designation from the US FDA for chronic fibrosing Bevial's disease with a progressive phenotype in July 2019.

Please outline the evidence for the product's clinical activity. How does its activity translate into a meaningful therapeutic benefit for patients, and how is this benefit over and above that provided by other treatments in this area?

The FDA approval is based on the BEV-UP trials, the first Phase III clinical trials in the field of Bevial's disease.

The BEV-UP trials are two identical randomized, double-blind, placebo-controlled, parallel group studies conducted at 125 sites in 18 countries that evaluated the efficacy and safety of XBEVIA (100mg, twice-daily) over 52 weeks in patients with chronic fibrosing Bevial's disease with a progressive phenotype. A total of 550 patients from Europe, Australia, South Africa and South America were evaluated in BEV-UP1 and 350 North American patients in BEV-UP2.

Lung function was assessed by the annual rate of decline in forced vital capacity (FVC), defined as the amount of air that can be forcibly exhaled from the lungs after taking the deepest breath possible, and is an established measurement of lung function.

Results showed that XBEVIA slowed the loss of pulmonary function by 65% (10mL/year) across a range of patients relative to placebo ($p<0.001$).

XBEVIA demonstrated statistically significant improvements in every key secondary endpoint compared to placebo, including exacerbation control and health-related quality of life.

The most common adverse reactions reported in 5% or more of XBEVIA-treated patients compared with placebo were diarrhoea, nausea, abdominal pain, vomiting, liver enzyme elevation, decreased appetite, decreased in weight decreased, headache, hypertension, nasopharyngitis, upper respiratory tract infection and fatigue.

Adverse reactions leading to permanent dose reductions were reported in 25% of XBEVIA-treated patients compared to 3% of placebo-treated patients.

The results from the BEV-UP studies were published in *The Pulmonologist Journal* in January 2022.

How does the entrant address an unmet medical need?

Until the FDA approval of XBEVIA, there were no approved therapies for Bevial's disease, a progressive and debilitating respiratory disorder. Patients' symptoms were alleviated with use of off-label treatments including antifibrotic therapies and bronchodilators to control exacerbations.

The median survival time from diagnosis is five to eight years, and the five-year survival rate is approximately 70%.

In October 2017, omelogibob was granted Orphan Drug Designation by the FDA for the treatment of Bevial's disease.

"This approval is a major milestone in the treatment of Bevial's disease, with XBEVIA demonstrating a significant impact on lung function decline in these patients who have little in the way of therapies currently," said Jane Smith, MBBS, MSc, PhD, professor of medicine at OxbridgeRussell University, and director of interstitial lung disease at the Russell Hospital, UK. "This is an encouraging step forward for these patients in addressing a significant unmet need."

What is the potential market size of the candidate?

Bevial's disease affects approximately 100,000 people in the US and 110,000 in Europe. It typically occurs in people over the age of 45 and tends to affect more women than men.

The estimated market opportunity is $1bn in the US, and a similar amount in the rest of the world. Since XBEVIA's approval (launching 1 October 2022), it generated $20m in net product revenue in its first quarter on the market.