

FDA Approves Apitegromab for Spinal Muscular Atrophy

The US FDA has approved apitegromab (Isembyld) for the treatment of spinal muscular atrophy (SMA) in adults and children aged 2 years and older who are receiving an SMN2-targeted therapy. The approval makes apitegromab the first therapy specifically targeting muscle loss associated with SMA, offering a new treatment approach alongside existing disease-modifying therapies.

Disease or Condition

SMA is a rare, progressive neuromuscular disease affecting approximately 1 in 10,000 live births and is among the leading genetic causes of infant mortality. It is caused by a faulty SMN1 gene that fails to produce a protein essential for motor neuron survival, leading to progressive muscle weakness and wasting.

A backup gene, SMN2, typically produces a broken version of this protein, and existing SMN2-targeted therapies work by correcting this defect so the body can produce enough of the missing protein to keep motor neurons alive. While these treatments have significantly improved outcomes for many patients, those with more advanced disease continue to experience substantial motor limitations (including the ability to walk or move independently), highlighting the need for therapies that directly address muscle loss.

Effectiveness

The effectiveness and safety of Isembyld were evaluated in a 52-week randomized, double-blind, placebo-controlled trial (NCT05156320). The trial enrolled 188 participants with SMA between age 2 to 21 years who were not able to move or walk independently. All participants were already receiving an approved SMN2-targeted treatment. Patients were randomly assigned to receive Isembyld 10 mg/kg, Isembyld 20 mg/kg via intravenous infusion, or placebo once every four weeks for approximately one year. The primary analysis was conducted in 156 patients aged 2 to 12 years.

The study measured change from baseline in the Hammersmith Functional Motor Scale Expanded, a standardized assessment of motor ability. Among patients aged 2 to 12 years, those receiving Isembyld 10 mg/kg showed a significant difference from those on placebo, with treated patients demonstrating improvement in motor function at one year while those on placebo declined. Those in the treatment arm were more than twice as likely than patients in the placebo arm to demonstrate a clinically meaningful improvement (34.2% vs. 13.5%).

Safety Information

The most common adverse reactions were upper respiratory tract infections, vomiting, cough, other viral infections, headache, gastroenteritis, and pharyngitis (sore throat). An increased risk of fractures, including serious fractures, was observed in patients treated with Isembyld. Isembyld may cause fetal harm and may affect reproductive function.

News Source:

https://medicaldialogues.in/neurology-neurosurgery/news/fda-approves-apitegromab-for-spinal-muscular-atrophy-179277?utm_campaign=&utm_content=US%20FDA%20clears%20Roche%27s%20Elecsys%20pTau217%20blood%20test%2C%20developed%20in%20collaboration%20with%20Lilly%2C%20to%20aid%20identification%20of%20amyloid%20pathology%20associated%20with---&utm_medium=email&utm_source=Newsletter&utm_term=