



The NHS will reimburse the first gene editing therapy for β -thalassemia and sickle cell anaemia
The AIFA Board of Directors approves the reimbursement of 7 new medicinal products (including 2 orphans), 6 generic medicines and 6 extensions of therapeutic indications

The first gene therapy with CRISPR-CAS9 technology authorised in Italy and Europe will be reimbursed by the National Health Service. At its meeting on 17 September, the AIFA Board of Directors approved the reimbursement of Casgevy (exagamglogene autotemcel), indicated for the treatment of β -thalassemia (TDT) and sickle cell disease (SCD), two serious inherited haemoglobinopathies caused by mutations in the β -globin gene. It is a highly innovative therapeutic path. Hematopoietic stem cells are extracted from the patient, genetically modified in the laboratory and re-infused into his/her body, where they give rise to blood cells capable of producing foetal (healthy) haemoglobin, reducing or eliminating the need for transfusions in TDT and vaso-obstructive seizures of SCD.

The other orphan drug to be reimbursed by the SSN is **Zynyz** (retifanlimab), a monoclonal antibody indicated as monotherapy for the first-line treatment of adult patients with Merkel cell carcinoma.

5 new molecules of chemical origin will be eligible for reimbursement:

- **Anzupgo** (delgocitinib) for the treatment of chronic hand eczema;
- **Camzyos** (mavacamten) for the treatment of obstructive hypertrophic cardiomyopathy (oHCM);
- **Krazati** (adagrasib) for the treatment of advanced non-small cell lung cancer;
- **Medepidol** (hydromorphone) for the treatment of severe pain in adults;
- **Vanflyta** (quizartinib) for the treatment of acute myeloid leukaemia (AML).

The AIFA Board of Directors also accepted reimbursement by the NHS of 6 generic medicines:

- **Apremilast G.L., Apremilast Krka and Apremilast Teva** (apremilast) for the treatment of psoriatic arthritis, psoriasis, and Behçet's disease;
- **Cyclophosphamide Seacross** (cyclophosphamide) for the treatment of different types of tumours and autoimmune diseases;
- **Diavic** (liraglutide) for the treatment of type 2 diabetes mellitus;
- **Intelence** (etravirine) for the treatment of human immunodeficiency virus type 1 (HIV-1) infection.

On the other hand, the extensions of therapeutic indications eligible for reimbursement concern 5 medicinal products already reimbursed for other indications: the humanised monoclonal antibody **Bimzelx** (bimekizumab), which will be reimbursed for two new indications; **Braftovi** (encorafenib) and **Mektovi** (binimetinib), two cancers indicated in combination for the treatment of non-small cell lung cancer (NSCLC); **Livmarli** (maralixibat), for the treatment of progressive familial

intrahepatic cholestasis (PFIC) and **Tepkinly** (epcoritamab) for the treatment of relapsed or refractory follicular lymphoma (FL).