Sickle cell gene therapy granted new 'innovation passport' under MHRA scheme to reduce the time to market for innovative medicines

bluebird bio's LentiGlobin™ for Sickle Cell Disease (bb1111) Gene Therapy *has been awarded an Innovation Passport Designation by the Medicines and Health products Regulatory Agency (MHRA)*

With a coordinated approval and reimbursement review, people with the rare blood disorder could benefit from the one-time treatment much sooner than by standard approval processes

Patients say they welcome the move to urgently bring new and innovative treatments to people with sickle cell disease

BASINGSTOKE, UK. 29th June 2021, 00.01 BST. A promising treatment for the rare blood disorder, sickle cell disease (SCD), is the latest to be awarded an 'Innovation Passport' under a new UK approval process to bring innovative medicines more rapidly to patients.

LentiGlobin™ for Sickle Cell Disease (bb1111), a gene therapy treatment developed by bluebird bio for adults with sickle cell disease, has been awarded an 'Innovation Passport' by the Innovative Licensing and Access Pathway Steering Group, consisting of the Medicines and Healthcare products Regulatory Agency (MHRA), National Institute for Health and Care Excellence (NICE) and the Scottish Medicines Consortium (SMC).

Launched in January this year, the passport aims to accelerate the treatment through regulatory approval and reimbursement, in a scheme known as the Innovative Licensing and Access Pathway (ILAP).[1]

The move may mean people with sickle cell can benefit from gene therapy much sooner than through standard, separate regulatory and reimbursement pathways.[1] If approved, LentiGlobin for Sickle Cell Disease (bb1111) could become the first one-time, potentially curative treatment for the rare blood disorder in the UK.

John James, Chief Executive of the Sickle Cell Society, commented: "We're thrilled to hear that another potential treatment option for sickle cell disease is on the horizon and wholeheartedly welcome the Innovative Licensing and Access Pathway Steering Group's efforts to accelerate regulatory approvals and reimbursement. Sickle cell is a chronic, life-long condition characterised by unpredictable pain crises which can require hospitalisation and have a significant detrimental impact on an individual's health and quality of life. That's why it's so essential that we continue to focus on

developing new tools and treatment options that enable patients to have more pain free days and a better quality of life."

Nicola Redfern, VP General Manager UK and Northern Europe Cluster Lead **of bluebird bio**, commented: "With this designation, the UK progresses another important step towards making gene therapy available for people with sickle cell disease. The designation clearly shows that the MHRA understands the value and enormous potential of innovative gene therapy for people with rare and serious inherited disorders such as haemoglobinopathies."

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About Sickle Cell Disease

Sickle cell disease is a rare blood disorder of the haemoglobin, the protein in red blood cells that is responsible for the colour of the cell and for carrying oxygen around the body.[2] The inherited genetic disorder affects around 15,000 people in the UK.[3] The main symptoms are anaemia and episodes of severe pain, caused by affected blood cells sticking together and blocking blood vessels – known as a 'sickle cell crisis'.[4] Over time people with sickle cell can experience damage to organs such as the liver, kidney, lungs, heart and spleen.[4] As a result, the condition may also shorten life-expectancy. In the UK, people with sickle cell disease have a mean life expectancy of 60 years[5] – compared to a national average of 78.7 – 82.7 years.[6] Currently, the only curative treatment is a bone marrow transplant; however, this is only suitable for a very limited number of individuals who have a matched donor.[7]

About LentiGlobin™ for Sickle Cell Disease (bb1111)

LentiGlobin™ for Sickle Cell Disease (bb1111) is an investigational gene therapy being studied as a potential treatment for sickle cell disease. bluebird bio's clinical development program for gene therapy for SCD includes the completed Phase 1/2 HGB-205 study, the ongoing Phase 1/2 HGB-206 study and the ongoing Phase 3 HGB-210 study.

LentiGlobin™ for Sickle Cell Disease (bb1111) received Orphan Medicinal Product designation from the European Commission for the treatment of SCD in April 2014, and Priority Medicines (PRIME) eligibility by the European Medicines Agency (EMA) in September 2020. The U.S. Food and Drug Administration (FDA) granted Orphan Drug status, Regenerative Medicine Advanced Therapy (RMAT) designation, and Rare Pediatric Disease designation for LentiGlobin for SCD. In June 2021, the MHRA granted LentiGlobin an Innovation Passport (IP) Designation which grants access to the Innovative Licensing and Access Pathway (ILAP).

LentiGlobin™ for Sickle Cell Disease (bb1111) Gene Therapy is investigational and has not been approved in any geography.

About bluebird bio

bluebird bio is pioneering gene therapy with purpose. From our Cambridge, Mass., headquarters, we're developing gene and cell therapies for severe genetic diseases and cancer, with the goal that people facing potentially fatal conditions with limited treatment options can live their lives fully. Beyond our labs, we're working to positively disrupt the healthcare system to create access, transparency and education so that gene therapy can become available to all those who can benefit.

bluebird bio is a human company powered by human stories. We're putting our care and expertise to work across a spectrum of disorders: cerebral adrenoleukodystrophy, sickle cell disease, β-thalassemia and multiple myeloma, using gene and cell therapy technologies including gene addition, and (megaTAL-enabled) gene editing.

bluebird bio has additional nests in Seattle, Wash.; Durham, N.C.; Zug, Switzerland; Milan, Italy; Utrecht, the Netherlands; Hampshire, United Kingdom; Paris, France; and Athens, Greece.

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