



UNITED KINGDOM THALASSAEMIA SOCIETY

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Dear Members,

It is with great sadness and disappointment that we share the following news with you.

Last week BlueBird Bio, the company that produces Zynteglo; a form of gene therapy for transfusion dependent thalassaemia decided to close its programme for severe genetic diseases (SGD), including thalassaemia and sickle cell disease in Europe.

Their statement said that their decision was driven by the challenges of achieving appropriate value recognition and market access for ZYNTGLO in Europe.

The company said “while European regulators have been innovative partners in supporting accelerated regulatory paths for these therapies, European payers have not yet evolved their approach to gene therapy in a way that can recognize the innovation and the expected life-long benefit of these product.”

The company has advised that despite them closing their European operations they are currently looking for a potential partner in the form a much larger pharmaceutical company who can help support them to provide their therapies in Europe.

We have been contacted by the National Institute for Health and Care Excellence (NICE) to say due to the company’s statement, NICE will be delaying their final decision and response of the outcome of the technology appraisal for Zynteglo being used as a possible curative treatment for transfusion dependent thalassaemia for in England and Wales. The final appraisal document (FAD) which would have contained the decision of the NICE committee on whether to recommend or not recommend this potentially curative treatment to be funded by the NHS was originally due to be published this week.

We are extremely heartbroken by the news and the implications this has to our thalassaemia community. However, we remain hopeful as the company searches for a partner and due to the ongoing trials and new treatment options for thalassaemia.

We would like to huge say thank you to our members and health care professionals who have supported us through the NICE technology appraisal and would also like to say thank you to some of bluebird bio’s employees who have fought, advocated, and supported us tremendously for thalassaemia.

We will continue our fight and advocacy for thalassaemia and will be speaking to NHS England about this in the coming weeks.

You can read the company’s press release here:

[https://investor.bluebirdbio.com/news-releases/news-release-details/bluebird-bio-reports-second-quarter-financial-results-and\(accessed\)](https://investor.bluebirdbio.com/news-releases/news-release-details/bluebird-bio-reports-second-quarter-financial-results-and(accessed))

16th August 2021

Living with Thalassaemia

A Charity Organisation · Registered charity number: 275107

Patrons: Ms Tonia Buxton; Mr Kypros Kyprianou, Mr Peter Polycarpou, Mr Adil Ray OBE and Ms Nina Wadia OBE