Regulators’ advice can make a difference for faster patient access to highly innovative therapies

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Early dialogue and frequent interactions between medicine developers, regulators, [health technology assessment bodies (HTAs)](https://www.ema.europa.eu/en/partners-networks/health-technology-assessment-bodies) and patients can play a key role in delivering robust data needed to enable accelerated access of patients to highly innovative therapies that address unmet medical needs. The chair of [EMA’s committee for advanced therapies (CAT)](https://www.ema.europa.eu/en/committees/committee-advanced-therapies-cat), Martina Schussler-Lenz, the chair of [EMA’s human medicines committee (CHMP)](https://www.ema.europa.eu/en/committees/committee-medicinal-products-human-use-chmp), Harald Enzmann, and the head of EMA’s [scientific advice](https://www.ema.europa.eu/en/glossary/scientific-advice) office, Spiros Vamvakas describe these interactions through the example of the recent authorisation of [Zynteglo](https://www.ema.europa.eu/en/medicines/human/EPAR/zynteglo" \t "_self) (lentiglobin) in a [perspective piece published in Clinical Pharmacology & Therapeutics](http://ascpt.onlinelibrary.wiley.com/doi/full/10.1002/cpt.1639?af=R).

Zynteglo is a gene therapy for the treatment of patients with beta-thalassemia, a rare inherited blood condition that causes severe anaemia. Some patients can be cured through a stem cell transplant from a healthy donor. However, patients who have no matched family donor miss out. For them, Zynteglo could be a game changer. Using a lentiviral viral vector, it adds functional copies of a modified β-globin gene into a patient’s own stem cells, thereby addressing the underlying genetic cause of the disease.

Groundbreaking therapies such as Zynteglo present specific challenges for those who assess their benefits and risks for the initial authorisation and those who assess their value as a basis for pricing and reimbursement decisions. Continuous dialogue throughout the development of the medicine, without compromising the impartiality of the assessment at the marketing-authorisation application stage, can help overcome these challenges.

In the case of Zynteglo, the medicine benefited from [PRIME](https://www.ema.europa.eu/en/human-regulatory/research-development/prime-priority-medicines), EMA’s platform for early and enhanced dialogue with developers of promising new medicines that address an unmet medical need and several interactions with EMA’s [scientific advice](https://www.ema.europa.eu/en/glossary/scientific-advice) office with input from patients’ representatives. These interactions led to a more robust application package to demonstrate the medicine’s benefits and risks, which allowed [accelerated assessment](https://www.ema.europa.eu/en/glossary/accelerated-assessment). In the article, the authors describe some of the specific clinical and manufacturing process issues which were identified and how these were overcome. They also show that early accelerated approvals are only possible if a robust post-approval plan is defined at [marketing authorisation](https://www.ema.europa.eu/en/glossary/marketing-authorisation) stage.

The [article](https://ascpt.onlinelibrary.wiley.com/doi/full/10.1002/cpt.1639?af=R) is accessible in open access in Clinical Pharmacology & Therapeutics.