bluebird bio Community Update

October 21, 2021

To the Patient, Caregiver, Advocacy and Healthcare Provider Communities for cALD and beta-thalassemia in Europe:

This past August, we announced that bluebird bio planned to discontinue its operations in Europe. When we set out to bring transformative gene therapies to patients and families, we were optimistic that a system that was eager for innovation would be prepared to ensure those who needed it most could have access. Instead, we encountered barriers to bringing these therapies to patients in Europe that have proven to be insurmountable. This is heartbreaking after more than a decade of work to develop potentially curative therapies, and years of collaboration with European patients, caregivers and study investigators throughout every step of the process.

The decision to cease operations in the EU and UK came after two years of discussions with European reimbursement authorities for ZYNTEGLO™ (betibeglogene autotemcel). During that time, it became clear that European authorities were unwilling to recognize the value of a one-time, potentially curative medicine. The reality is that it would cost bluebird more to deliver these therapies to patients than what European authorities were willing to pay. We simply could not sustain operations in Europe without gaining sufficient reimbursement for ZYNTEGLO across multiple countries in a reasonable timeframe.

Since our last update, despite our best efforts, we have been unable to identify a partner to continue the commercialization of our gene therapies in Europe. Therefore, as part of the winding down of operations, we made the difficult decision to withdraw the regulatory marketing authorization for SKYSONA™ (elivaldogene autotemcel) from the European Union, and our marketing application for SKYSONA from the Medicines and Healthcare products Regulatory Agency (MHRA) of the United Kingdom (UK). We anticipate withdrawing marketing authorizations for ZYNTEGLO from both the EU and the UK by early 2022.

We are committed to the long-term follow-up of patients previously enrolled within the EU clinical trial programs as planned, but we will not initiate any new clinical trials in Europe for our therapies for beta-thalassemia, cerebral adrenoleukodystrophy or sickle cell disease. While we continue to investigate ways to make these therapies available to patients outside of the U.S., the path forward is unclear.

This decision was difficult for all of us at bluebird, who believe in these therapies and want nothing more than to make them available to patients. We recognize this news is also extremely disappointing to all of those who contributed to the development of these gene therapies and most importantly, to patients with cALD and beta-thalassemia and their families.

On behalf of all of us at bluebird, I want to express our sadness at this decision and deep appreciation for your support.

With gratitude,
Andrew Obenshain
president, severe genetic diseases, bluebird bio