



bluebird bio therapies approved by the U.S. Food and Drug Administration (FDA) are one-time gene therapies. These gene therapies are only approved for use in the U.S.

bluebird recognizes there is a significant unmet need among patients living with severe genetic diseases outside of the U.S. This document is intended to answer questions from patients and caregivers residing outside of the U.S. on FDA approved bluebird bio gene therapies.

Why are bluebird bio gene therapies only available in the U.S.?

bluebird received approval from the European Medicines Agency (EMA) in May 2019 for a one-time gene therapy. Following prolonged negotiations with reimbursement authorities in both Europe and the UK, bluebird was unable to reach an agreement that would enable European patients access to the therapy and as a small company, it could not sustain the cost of staying in Europe. In August 2021, bluebird made the decision to exit Europe. At this time, bluebird does not have plans to bring its gene therapies back to Europe or to pursue regulatory approval in other countries outside of the U.S.

How can I access bluebird bio gene therapies if I live outside of the U.S.?

bluebird gene therapies are available exclusively through a network of bluebird Qualified Treatment Centers (QTC), specialized hospitals in the US that are trained to administer gene therapy. Patients who reside outside of the US and are seeking treatment with a bluebird gene therapy will need to be treated through a Qualified Treatment Center.

How can I find a Qualified Treatment Center?

Please contact bluebird's patient services program, *mybluebirdsupport*, at 833-888-NEST (833-888-6378) or <https://www.mybluebirdsupport.com> for the most up to date list of bluebird Qualified Treatment Centers in the U.S.

How can I get support paying for bluebird bio gene therapy if I live outside of the U.S.?

Since bluebird gene therapies are only approved in the U.S., patients traveling from outside of the U.S. will be responsible for the cost of the treatment themselves. In many cases, accessing bluebird gene therapy in the U.S. will require patients to pay out of pocket for the cost of the therapy and ancillary costs of pursuing treatment. Some examples of ancillary treatment costs include those related to evaluation and screening, hospitalization, and fertility preservation. Please note this list is not exhaustive.

In some instances, reimbursement authorities from the patient's home country may be willing to provide financial support for patients seeking gene therapy in the U.S. Those interested in learning more should speak with their treating physician and reimbursement authorities in their home country, as well as the U.S.-based Qualified Treatment Center where they are seeking treatment with gene therapy.



Is bluebird offering any support for patients who travel from outside of the U.S. to receive bluebird gene therapy?

bluebird is not able to offer financial support to patients who are not U.S. residents.

Are there any organizations that can provide support for gene therapy?

Nonprofit and patient advocacy organizations may be able to provide additional information and educational resources related to gene therapy. The following are a few organizations focused on rare disease. Please note this list is not exhaustive and we are providing it for reference purposes only. bluebird is not affiliated with these organizations and has no influence or involvement in the donation or distribution of funds administered. We do not endorse organizations or the content of their websites.

- ALD Alliance <https://www.aldalliance.org/>
- ALD Connect <https://aldconnect.org/>
- Be The Match <https://bethematch.org/>
- Cooley's Anemia Foundation <https://www.thalassemia.org/>
- Global Genes <https://globalgenes.org/>
- National Organization for Rare Disorders (NORD) <https://rarediseases.org/>
- Thalassemia International Federation <https://thalassaemia.org.cy/>
- The Assistance Fund <https://tafcares.org/>

For more information, including how to find a qualified treatment center, contact *mybluebirdsupport* at 833-888-NEST or www.mybluebirdsupport.com