bluebird bio marks Rare Disease Day 2021 with launch of patient testimonial video: 'Living with Thalassaemia in Europe: Stories from patients of different ages'

Personal stories from 9 patients from 7 European countries, aged between 4 and 61, shed light on the impact of living with thalassaemia

ZUG, Switzerland— Feb. 28, 2021— To mark Rare Disease Day 2021, gene therapy company bluebird bio has joined forces with a number of leading patient organisations and clinicians to highlight the personal stories of European patients who are living with the rare inherited blood disorder thalassaemia.

Nine patients from seven countries (Italy, France, Germany, Greece, Cyprus, Belgium, and the Netherlands), each representing different life stages between the ages of 4 and 61, feature in a 11-minute video, together with national patient group representatives and quotes from leading clinicians.

Filmed during the constraints of the COVID-19 pandemic, the patients' stories are told through a mix of selfie videos, still photography and animated facts, and convey their day-to-day perspectives of living with the condition in different daily scenarios such as school, work, and leisure time. The film will be hosted on bluebirdbio.eu and shared on the social media channels of bluebird bio and those of participating patient groups.

"Thalassaemia is one of the most common inherited blood disorders in the world affecting 1 in 100,000 people across the world and 1 in 10,000 people in Europe,[1]" explained Nicoletta Bertelli, European Thalassaemia Patient Advocacy Lead at bluebird bio." It is especially prevalent in countries like Italy, Greece and Cyprus with fewer cases in Germany, the Netherlands, Belgium and the Nordics."

Although there are many cases of Thalassaemia among people of Mediterranean, Middle Eastern, South and East Asian and African descent, global population movements mean that the disease now occurs across the world including in northern and western Europe. This calls for health policy makers across Europe to address the health and care needs of affected groups as part of their national health plans.

Nicoletta Bertelli continued: "Beyond the immediate patient community and specialist clinicians, both the disease and its impact on quality of life, are poorly understood. We decided to collaborate with patient groups to commission this video to raise awareness about what this disease really means. Despite the advances in management and care which have made it possible for patients to live longer, they still face physical and medical challenges, and life expectancy is lower when compared to the general population."

People with thalassaemia have a genetic mutation which affects their red blood cells. This can cause severe anaemia and the need for frequent, lifelong blood transfusions and iron chelation therapy to remove the build-up of iron in their bodies. Side effects from this treatment can cause complications such as organ damage, osteoporosis and problems with the heart, liver and endocrine system resulting

[1] Galanello and Origa, Beta-thalassemia, Orphanet Journal of Rare Diseases 2015, 5:11

in reduced life expectancy.[2345]

Although treatments for thalassaemia have improved over the past few decades enabling some patients to live into their fifth decade and some even beyond,[6] the experience of living with the disease still presents many challenges. These are placed into focus by the six patient testimonies that feature in the video: *'Living with Thalassaemia in Europe: Stories from patients of different ages'*.

Common challenges raised by the patients include:

- Adjusting to the disease during childhood, missing school, making friends, and being perceived as 'weak'
- Coping with lifelong blood transfusions, iron chelation therapy which means being connected to a pump for several hours a day, continuous injections, and regular visits to the hospital
- Not being able to enjoy hobbies fully and be as physically active as 'normal' people
- Holding down a job, as they get older, given the constraints imposed by regular treatment and the fatigue caused by anaemia
- The need to always consider their thalassaemia first and foremost and adjust life accordingly
- Seeing friends with thalassaemia die prematurely
- Dealing with mental health issues and depression
- General low awareness and understanding of the condition

Mr. Panos Englezos, President of the Thalassaemia International Federation, Cyprus, who participated in the video, commented that: "I was eager to take part in this project to shine a light on the thalassaemia community and to help foster a greater understanding about this condition and what it really means for quality of life of patients."

"The stories of these patients are truly inspiring, and it is tremendous to see the courage and optimism they display in living their lives, in spite of the disease. But, as a parent who lost their child to thalassaemia, I would not wish this experience on anyone. I hope with all my heart that one day this serious disease will be a relic of the past".

But patients need to work hard to manage their condition, adhering to a lifelong treatment regimen and relying on blood transfusions and blood donations to survive.

Their stories are inspiring, but show they still have unmet needs.

#ChallengeThalassaemia

---

[2] TIF. Living with Transfusion-Dependent β-Thalassaemia (TDT). 2021. [ONLINE] Available at: https://thalassaemia.org.cy/news/living-with-transfusion-dependent-%CE%B2-thalassaemia-tdt/ Last accessed February 2021

[3] Guidelines for the Management of Transfusion Dependent Thalassaemia (TDT). 3rd ed. Thalassaemia International Federation. 2014. ISBN-13:978-9963-717-06-4.

[4] Baronciani D, Angelucci E, Potschger U, et al. Hematopoietic stem cell transplantation in thalassemia: a report from the European Society for Blood and Bone Marrow Transplantation Hemoglobinopathy Registry 2000–2010. Bone Marrow Transplant. 2016;51(4):536-541.

[5] Ladis V, Chouliaras G, Berdoukas V, et al. Survival in a large cohort of Greek patients with transfusion-dependent beta thalassaemia and mortality ratios compared to the general population. Eur J Haematol. 2011;86(4):332-8.

[6] Shamshirsaz, A.A., Bekheirnia, M.R., Kamgar, M. et al. Metabolic and endocrinologic complications in beta-thalassemia major: a multicenter study in Tehran. BMC Endocr Disord 3, 4 (2003).

Note to editors

About: 'Living with Thalassaemia in Europe: Stories from patients of different ages'

- This video project was funded by bluebird bio and realized thanks to the support and input of ALT Ferrara (Italy), DEGETHA & FRIENDS (Germany), the Hellenic Thalassemia Association (ESTHA, Greece), Oscar (Netherlands), the Pancyprian Thalassaemia Association (Cyprus), SOS Globi (France) and a special guest appearance from the Thalassaemia International Federation (Cyprus), all of whom assisted in the identification of the patients who have kindly shared their stories.
- It was supported by a number of leading healthcare professionals who are quoted in the national versions of the video. They include Prof. Frédéric Galactéros, Hôpitaux Universitaires Henri Mondor, France; Dr. Soteroula Christou, Cyprus Thalassaemia Centre, Cyprus; Dr. Ferras Alashkar, Universitätsklinikum Essen, Germany; Dr. Carmen Aramayo-Singelmann, Universitätsklinikum Essen, Germany, and Prof. Gian Luca Forni, President of SITE (Italian Society for Thalassemia and Hemoglobinopaties), Galliera Hospital, Italy.
- The video is available in a pan EU version in English featuring six patients, and in local language versions in Italian, French, Greek, German, and Dutch. These also include a number of in-depth native patient testimonies.

About bluebird bio, Inc.

bluebird bio is pioneering gene therapy with purpose. From our Cambridge, Mass., headquarters, we're developing gene 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 including cerebral adrenoleukodystrophy, sickle cell disease, β-thalassaemia and multiple myeloma, using three gene therapy technologies: gene addition, cell therapy and (megaTAL-enabled) gene editing.

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

For further information, visit bluebirdbio.eu

Media:
Claudia Nabaie, +41-79-906-5814
cnabaie@bluebirdbio.com