The only innovative treatment for CALD will not be available in Europe : the incomprehension of ELA International!

Press release – February 3rd 2022

Authorised by the European Medicines Agency in 2021, Skysona, the first innovative treatment for cerebral adrenoleukodystrophy (CALD), will not be marketed in Europe. The Bluebird Bio laboratory that was to produce it is closing its doors in Europe, condemning our children. Why?

Cerebral adrenoleukodystrophy (CALD) is a rare and dreaded genetic disease that mostly effects children under the age of 10 and destroys the central nervous system (brain and spinal cord) within a few months. As a result, a previously healthy child gradually loses all of his or her functions (sight, hearing, motor skills, speech, memory, etc.). The only hope for survival is to intervene before the first symptoms appear by detecting it and treating the child as soon as possible. However, if a treatment exists, it is not accessible: the laboratory that was supposed to produce it has decided to leave Europe.

Since 1992, the ELA association has been promoting research on CALD

Since its creation in 1992 in France, the ELA association has been working to find treatments for this disease. We, the parents of sick children, supported the team of Professor Patrick Aubourg's and Doctor Nathalie Cartier (PARIS - Hôpital Saint Vincent de Paul, later Hôpital Bicêtre) and financed the pre-clinical studies and the first clinical trial of gene therapy. Four young patients received bone marrow autografts to correct the problematic gene. This innovative technique was praised in 2009 by leading scientific journals such as "Science" and "Nature". This initial success, which holds out hope for other diseases, has paved the way for a second, larger trial by the American biotech company Bluebird Bio. The hope for the families was immense.

In 2021, ELA's determination will finally be rewarded!

ELA's continuous efforts over the past 30 years, with €47 million invested, have boosted international research on leukodystrophies. We were proud to announce in July 2021 that the European Medicines Agency (EMA) had authorised the first innovative treatment for CALD: Skysona, developed by Bluebird Bio.

A heavy blow in 2022: the laboratory leaves Europe. The treatment will not be distributed.

While marketing authorisation is usually the decisive step in making the treatment available to patients, Skysona is still not distributed in Europe. This is likely to continue as the laboratory concerned has closed all its offices. **Why?** To date, we have no explanation from either the laboratory or the Medicines Agency. **Is it a problem of cost?** This type of treatment is expensive, as it takes a long time to develop (it has been 15 years since the first child was treated) and biotech investments are risky. But this is not a drug to be taken throughout a lifetime, a single dose is enough!

We ask the health authorities and the laboratory to resume negotiations as soon as possible in order to end the deadlock and finally treat the sick children.

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