

**Thalassemia** is a terrible disease common all over the world: according to the last report of the World Health Organization (WHO), 330 thousands children are born each year with thalassemia and only 3 thousands have adequate transfusions.

In Sicily there are 2,000 patients with thalassemia and the number of heterozygous carriers is approximately 7% of the population.



From the age of 6 months, patients with *thalassemia major* need to receive blood transfusions every 15 days and chelation through the administration of the subcutaneous or oral drugs every single day. Their life expectancy is reduced by severe complications as the natural development of their disease includes a severe iron overload with damage to several organs such as the liver and heart as well as the endocrine system and bones. To date, there is no definitive cure apart from familial or non-familial bone marrow transplantation. These procedures involve a very high mortality rate because of differences between recipient and donor antigens. Gene therapy treatment is the most likely option if research is to find a safe cure for this disease.

The Piera Cutino Onlus Association, a Sicilian non-profit organization based in Palermo, together with the Center for Cell Engineering and the Center for Stem Biology at the Memorial Sloan-Kettering Cancer Center (MSKCC) in New York (USA), are developing a project for a definitive cure from thalassemia. The project is aimed at modifying the stem cells of these patients through the use of a lentiviral vector. The genetically modified stem cells will be auto-transplanted to these patients after mild myelo-ablation.

Ten patients, many of whom are Sicilians, will undergo transplants. The transplant of all ten patients requires resources for € 2.5 million over two years. The major cost for the treatment of these patients is the production of the lentiviral vector (\$100,000 for each patient). Moreover, the treatment includes peripheral stem cell mobilization, chemotherapy treatment, blood support and the treatment of complications.

The Piera Cutino Onlus Association are appealing for donations. They hope that large Italian federation in the States will support the gene therapy research, by sending a group order of Christmas greeting cards with Typical Sicilian land-scapes.

If a minimum of 300 members feel they can make a minimum donation of 15 euro each, the Piera Cutino Association will send each member one card personalized with his name and their logo to highlight the social value of the donation and two cards without his/her name.

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The minimum number of cards ordered is 300 personalized and 600 without members' names. For details of how to order the cards, Associations should contact the Piera Cutino via e-mail ([sergio.mangano@pieracutino.it](mailto:sergio.mangano@pieracutino.it) or [thalassemia@pieracutino.it](mailto:thalassemia@pieracutino.it)).

70% of funds raised thanks to such donations will be used to support the cost for lentiviral production and for the treatment of the patients involved in this trial at the MSKCC itself. The other 30% will go towards a new center for Thalassemia that the Association is building at the "Villa Sofia – Cervello" Hospital of Palermo, which will be structured as follows:

1. a plan for hospital admission, with day hospital and beds;

2. a floor dedicated to scientific research, with new laboratories and highly qualified personnel;
  
3. a structure to be used to house the families of patients of the entire hospital;

The building of the Hospital Area and "Home Hotel" was completed last September (as shown in the photo on the left). Now the main goal is to complete the construction of research laboratories.

Details of this and other projects that the Association promotes can be found on their website at [www.pieracutino.it](http://www.pieracutino.it) where their latest financial statements (12.31.2011) are also posted. Federations who support the initiative with a group order of greetings cards would be given the chance to see the development of the research laboratories: the Piera Cutino Onlus would be happy to host a delegation of their members in the "house hotel" that will soon be operational on the top floor of the new center.

Naturally, individuals and smaller group can send donations, without ordering the card, direct to the Piera Cutino Association (Banca Intesa San Paolo, Filiale di Palermo Via Roma 495, IBAN number: IT19 M0306904600 100000008081, BIC/SWIFT code: BCITITMM or by credit card via the website [www.pieracutino.it](http://www.pieracutino.it)

We invite our readers to visit this website so that they can see what Sicilians can achieve when they work together to solve the problems of their island.

Source: Times of Sicily