

Nature publishes results of gene therapy treatment in phase 1/2 beta-thalassemia study

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bluebird bio (formerly Genetix Pharmaceuticals Inc.) today announced publication in the journal *Nature* of its promising Phase 1/2 data highlighting positive results of LentiGlobin gene therapy treatment in a young adult with severe beta-thalassemia, a blood disorder that is one of the most frequent inherited diseases.

The patient, who had been transfusion dependent since early childhood, has become transfusion independent for the past 21 months - more than two years after treatment with the LentiGlobin vector. The study also identified a subset of cells with the corrected beta-globin gene that overexpressed a truncated form of a gene called HMGA2. The patient has not experienced any adverse events. The data show that while early on, the HMGA2 clone was a significant portion of the corrected cells, the clone levels had declined at the time the paper was prepared, and further follow up indicates the decline is continuing.

"Although based on the first treated patient, we believe these results are impressive and illustrate for the first time the significant potential for treatment of beta hemoglobinopathies using lentiviral beta-globin [gene transfer](#) in the context of autologous [stem cell transplant](#)," said Philippe Leboulch, M.D., senior author of the study and head of the Institute of Emerging Diseases and Innovative Therapies of CEA and INSERM; professor of medicine, University of Paris; and visiting professor, Harvard Medical School. "For beta-thalassemia, we have worked intensely for almost 20 years to design, develop and manufacture LentiGlobin to provide a sustained high level hemoglobin production,

resulting in a major clinical benefit. It has been very rewarding to follow this patient as his life has dramatically improved since receiving our treatment."

"For the first time, a patient with severe beta-thalassemia is living without the need for transfusions over a sustained period of time," said Marina Cavazzana-Calvo, M.D., first co-author of the study and professor of hematology, University of Paris and chief of Cell and Gene Therapy Department, Necker-Enfants Malades Hospital in Paris. Salima Hacein-Bey-Abina, Ph.D., professor of immunology, University of Paris, added, "These results are not only important due to the tremendous medical need that exists for thalassemia patients around the world, but also represents a significant step forward for the field of autologous stem cell therapy as an emerging therapeutic modality."

Dr. Françoise Bernaudin, the clinical hematologist who has followed this patient since early childhood, said, "It is wonderful to see that this young man is for now free of transfusions and injections for iron chelation. He is happy to have a normal life back, and for the first time has a full-time job as a cook in a main restaurant in Paris. We are now even able to bleed him regularly to help remove toxic iron that had accumulated over the years because of blood transfusions."

The paper, titled "Transfusion independence and HMGA2 activation after gene therapy of human beta-thalassemia," is available in the online publication of *Nature* at www.nature.com.

"We believe the human findings in beta-thalassemia, as well as the recently published data in *Science* on two patients with childhood cerebral adrenoleukodystrophy (CCALD), highlight the significant opportunity for bluebird bio's gene therapy platform to help patients with severe genetic disorders," said Nick Leschly, president and CEO of bluebird bio. "We are committed to building a world-class company in

gene therapy led by outstanding people as we move aggressively forward with multiple clinical studies, including our ongoing clinical trials for the development of LentiGlobin for beta-thalassemia and our product for CCALD."

Provided by Pure Communications Inc.

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