**Headline**

## Genethon in talks for biopharma gene therapy manufacturing collaborations – CEO

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Genethon, the France-based non-profit organisation, is in talks with biopharmaceutical firms involved in the gene therapy space for manufacturing collaborations at its new facility, expected to completed by year end, said Frédéric Revah, CEO at Genethon.

This gene therapy manufacturing site, Bioprod Généthon, will be the largest of its kind in Europe and the US once assembled, the CEO said.

Genethon is looking seriously at partnership deals with these firms and is likely to enter a deal where there is a risk share, with some financing, said Revah. Such collaborations would involve Genethon helping these companies to set up their manufacturing processes for these complex gene therapies, from "concept to clinic," he explained. The field of gene therapy is still relatively new, and there have not been many partnerships of this kind yet, Revah noted.

Genethon is 80-90% funded by the French Association against Myopathies (FAM) and is also in the early stages of developing its own gene therapies. Yesterday, Genethon announced the launch of a third centre for its Phase I/II 15 patient trial investigating its gene therapy for Wiskott Aldrich Syndrome (WAS), a rare primary immune deficiency disease causing significant bleeding due to low platelet count and increased incidence of serious infections. Treatment is based on ex vivo gene transfer, whereby a lentiviral vector carrying the therapeutic gene is inserted into the patient's hematopoietic stem cells.

The multicentre trial is being run in Paris at Hôpital Necker-Enfants Malades, in London at Great Ormond Street Hospital and in Boston at the Children's Hospital Boston. The firm hopes to recruit four patients by the end of the year - the London and Paris centres are currently treating one patient each, and there are patients lined up in Boston, Revah said. Genethon expects to recruit five patients next year, he added.

The trial will look at all safety and efficacy parameters he noted, adding that if efficacy is established from this trial, the company will be very quick to apply for temporary allowance of use in Europe which will allow commercialisation.

by Abigail Moss



**Company:**

Genethon

**Company**

Genethon

**Topic**

CROs, CMOs, EDC
Licensing
Other
Product Development

**Intelligence Grade**

Strong evidence

**Sub-sectors**

Drug development

**Country**

France

**Contacts**

Frederic Revah

Update Data