

► therapy has been approved for routine use by the US Food and Drug Administration or the European Medicines Agency. That situation must change, according to the board of directors of the French Muscular Dystrophy Association, which created Genethon in 1990 and has funded it ever since through its annual telethons.

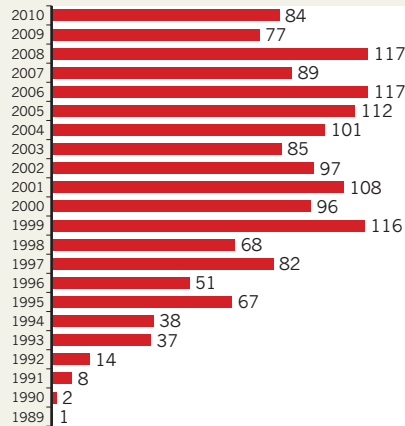
BROAD POTENTIAL

Although the best-known gene-therapy trials have been done in children with immune deficiencies, the technique could tackle a much wider array of diseases. Most of the current clinical studies are in cancer, with researchers trying to introduce genes that will kill the cancer cells directly, or prod the patient's immune system into attacking them. And on 6 January, the American Society of Gene and Cell Therapy sent the director of the US National Institutes of Health a list of the diseases it believes will benefit most in the next six years from investment in translating basic research to the clinic. It included rare immunodeficiency and eye disorders, as well as more common blood disorders, two cancers and Parkinson's disease.

Mavilio worked on the world's first gene-therapy trial, which treated children with the immunodeficiency disorder ADA-SCID at the San Raffaele Scientific Institute in Milan.

GENE PROMISE

Dozens of gene-therapy clinical trials are now approved by regulatory authorities every year.



Another 141 trials have been approved at unspecified times.

Pharmaceutical giant GlaxoSmithKline last year forged a multi-million-euro alliance with the institute to develop similar gene therapy for rare diseases, becoming the first pharmaceutical giant to invest significantly in the field.

“The disadvantage of Genethon compared to the San Raffaele is that it does not have its own hospital,” Mavilio says. “We will be very

proactive in forging collaborations with top clinicians in Europe and beyond so that we can become a major hub for gene-therapy networks.”

The vector-production facility will be a huge asset for this, he says. “But it won't be enough. To get good collaborations, we also have to be known as a force in science.” Genethon moved away from basic research in 2006, to focus on vector production. It now gets more than 90% of its money from the Telethon, but Mavilio wants this to be significantly supplemented by competitive research grants.

Philippe Moullier, director of the French national biomedical research agency's gene-therapy unit in Nantes, and a Genethon adviser, warns that the firm needs “to remain humble and move slowly — I don't know if we can demand to become a European hub”. Thrasher, at least, is enthusiastic about the institute's ambitions, predicting that “Genethon will probably become our first port of call”. ■

CORRECTION

The photo caption in the News story ‘Gemini's twin telescopes reboot’ (*Nature* **481**, 251; 2012) incorrectly identified the Gemini North telescope as Gemini South.