

Gene therapy continues to advance

FDA's Advisory Committee unanimously recommends that Luxturna from the American gene therapy company Spark Therapeutics will be approved for the treatment of an unusual form of eye disease called retinitis pigmentosa

Retinitis pigmentosa is a genetic disease. Symptoms comprise of, among other things, involuntary eye movements, tunnel vision and loss of night vision. The disease can lead to complete blindness. No effective treatment has previously existed, but the recommendation from the FDA's Advisory Committee probably means that the FDA (the Federal Drug Administration) will approve Luxturna for use on the American market.

If Luxturna obtains final approval from the FDA, it will be the first approved gene therapy treatment for a genetic disease in the USA. The treatment can potentially be administered once and have a lifetime effect on the patient.

Luxturna has carried out four years of clinical studies. No gene therapy-related side-effects have been identified. A few side-effects have arisen in connection with the surgical operation through which the gene therapy is administered.

The recommendation from FDA's Advisory Committee to approve Luxturna is another great success for gene therapy of late. In August 2017, the FDA approved Kymriah from the pharmaceutical company Novartis for the treatment of certain paediatric patients and young people with a treatment-resistant type of acute lymphatic leukaemia.

To Sparks pressrelease:

<http://ir.sparktx.com/phoenix.zhtml?c=253900&p=irol-newsArticle&ID=2306441>

The National Board of Health and Welfare (Socialstyrelsen):

<http://www.socialstyrelsen.se/ovanligadiagnoser/leberskongenitalaamauros>

About CombiGene AB

By combining modern neuroscience with recent advances in gene delivery, CombiGene has developed a method shown to suppress epileptic seizures in preclinical studies. The current focus is on continuing to develop this method into an effective and safe therapy for epilepsy patients, but the method may also have development potential as a means of treating other neurological disorders. Founded on the basis of scientific discoveries made at Lund University and the University of Copenhagen, CombiGene has offices at Medicon Village in Lund, Sweden. The company is public and listed on the Swedish marketplace AktieTorget. www.combigene.com

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