

European regulators back western world's first gene therapy drug

European regulators have recommended approval of the Western world's first gene therapy drug — after rejecting it on three previous occasions — in a significant advance for the novel medical technology.

More than 20 years since the first experiments with the ground-breaking method for fixing faulty genes, scientists and drug companies are still struggling to apply gene therapy in practice.

Friday's decision by the European Medicines Agency (EMA) is a win for the drug's maker, the small Dutch biotech company uniQure, and a potential lifeline for patients with the ultra rare genetic disorder lipoprotein lipase deficiency (LPLD).

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