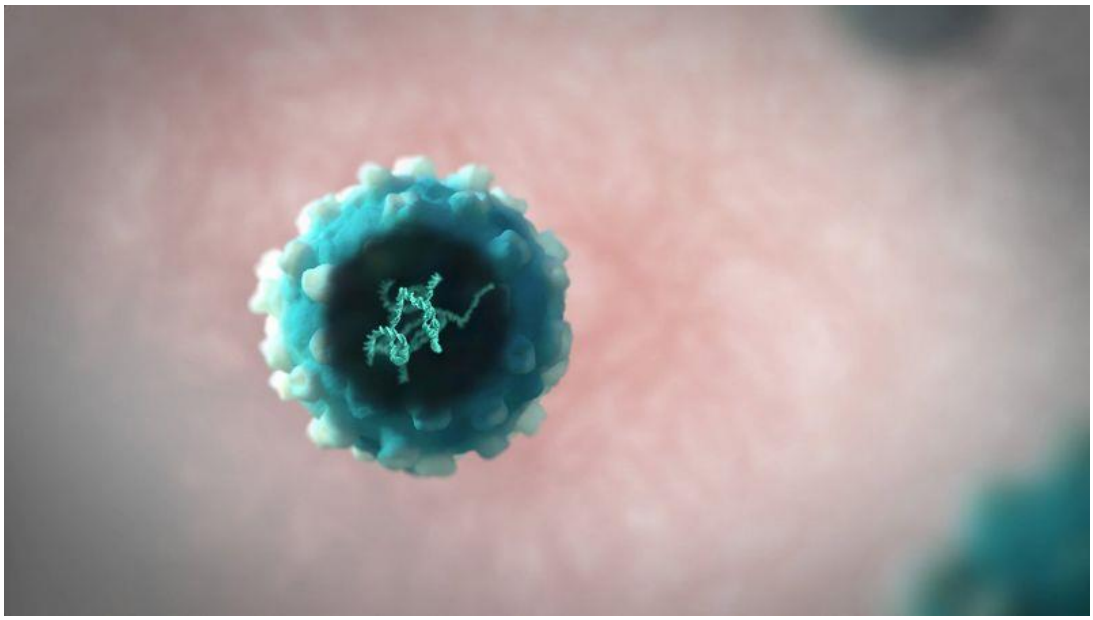
Europe approves 1st gene therapy for children



Grant Thompson/GSK via Reuters

This undated photo shows a vector, which is used to transport healthy genes into patients' cells.

2:20 pm, April 05, 2016

ReutersLONDON (Reuters) — The world's first life-saving gene therapy for children, developed by Italian scientists and GlaxoSmithKline, has been recommended for approval in Europe, boosting the pioneering technology to fix faulty genes.

The European Medicines Agency (EMA) said last week it had endorsed the therapy, called Strimvelis, for a tiny number of children with ADA Severe Combined Immune Deficiency (ADA-SCID) for whom no matching bone marrow donor is available.

Around 15 children a year are born in Europe with the ultra-rare genetic disorder, which leaves them unable to make a type of white blood cell. They rarely survive beyond two years unless their immune function is restored with a suitable bone marrow transplant.

SCID is sometimes known as “bubble baby” disease, since children born with it have immune systems so weak they must live in germ-free environments.

Strimvelis is expected to secure formal marketing authorization from the European Commission in a couple of months, making it the second gene therapy to be approved in Europe, after UniQure’s Glybera, which treats a rare adult blood disorder.

Research into gene therapy goes back a quarter of a century but the field has experienced many setbacks, including some disastrous clinical trial results in the late 1990s and early 2000s. Now, though, optimism is building, helped by the discovery of better ways to carry replacement genes into cells.

Martin Andrews, head of GlaxoSmithKline’s rare diseases unit, believes the technology is proving itself, although it remains at an early stage of development. “We’re on page one of chapter one of a new medicine text book,” he told Reuters.

A host of challenges still need to be overcome, including the complexity of delivering a product like GSK’s new treatment, which requires bone marrow cells to be taken from the patient, processed and injected back.

Trickiest of all may be pricing, given the tiny market for a therapy like Strimvelis. GSK is not putting a price on its product but a source close to the company said that, if approved, Strimvelis would cost “very significantly less than $1 million.”

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