Is gene therapy research for single-gene diseases at risk under Trumpcare?

[When] the House of Representatives passed the American Health Care Act of 2017,…*DNA Science* addressed the possibility of the AHCA forcing pregnant women to carry doomed fetuses to term, the discussion now in the hands of 13 senators…[Now] I fear for the treatments for single-gene conditions, both the short-term and available protein-based ones as well as the not-yet-approved gene therapies.

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CNN.com once told the remarkable story of recent college grad Ryan Dant., [who was diagnosed with] a form of mucopolysaccharidosis (MPS) [at age 3]…Ryan wasn't expected to survive beyond age 10, but entered a clinical trial for an enzyme replacement therapy (ERT). It won FDA approval in 2003, [but it is very expensive].

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The high cost of lifelong frequent infusions or injections of ERT is why the "forever fix" of a gene therapy is an attractive alternative, even if a booster or two becomes necessary. Gene therapy delivers the DNA instructions for making the missing enzyme. Another reason to seek gene therapy (or editing) is that enzyme infusions don't reach the brain.

Theoretically, gene therapy should be more economical than ERT, once research costs have been recouped…Yet the first FDA approval for a gene therapy has yet to happen.

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: Will Short Term and Long Term Treatments for Single-Gene Diseases Survive?