3 promising CRISPR therapies, including one targeting Duchenne Muscular Dystrophy

With dozens of efforts in the making, from premature aging to obesity and developmental brain disorders, here are the frontrunners beyond CRISPR-based cancer therapy to watch out for.

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In February 2019, a team found that a single injection of the CRISPR machinery in mice with [Duchenne Muscular Dystrophy] boosted their muscle function for well over a year.

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Hot on DMD's heels is a CRISPR-based therapy that hopes to eliminate—deep breath—Leber's congenital amaurosis type 10. Known as LCA10, it's the most common form of inherited blindness in children. [December 2018], Editas Medicine, along with its partner Allergan, received the FDA green light to start pushing for a phase 1/2 trial

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Scientists are using CRISPR to give [sickle cell anemia] patients' blood stem cells a protein called HbF, which is a natural protein present at birth that is especially potent at carrying oxygen, which "sickled" red blood cells struggle with.

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Of these three non-cancer CRISPR therapies, the first to hit the market will mark a historical turning point for gene therapy and for medicine as a whole: to efficiently edit the very base code that makes us who we are.

Read full, original post: <u>The Three Frontrunners in the CRISPR Therapy Race</u>