There are a few crisper stocks/gene therapy stock I have studied.

Crsp, ntla, and edit. Sangoma  and caribou biosciences. Edit is dogshit and probably won’t make it, I haven’t studied sang or caribou yet, who are farther behind than crsp and ntla. Crisper tech is a way to insert DNA into peoples genes. Revolutionary tech first found in bacteria and now used by humans. Doudna and others won a nobel prize for their work on this.

So everyone was hyped hyped for this tech and the stocks were traded at inflated market caps of 18B during bubble times still nothing has been approved yet. There are limitations to this tech to get the dna inserts into anything that isn’t the liver or bone marrow stem cells. To get the dna to insert in the right place with high-fidelity is also a challenge. CRISP understands these limitations 1000% better than the rest and is a reason why EDIT with their ocular drug that needed to perfectly insert a functional gene into the eye is stumbling right now.

CRSP also is tackling the bigger problems rn with 100K sickle cell disease patients in the US, (10K in UK, 10K France, don’t know GER or ITL). They are the pharma company that most closely models natures at this point. They iterate better than everyone else. Next drug up ctx110 for end of life, out of options (salvage therapy) for high-grade B-cell lymphomas shows complete response for months up to a year. Keytruda and PPAR inhibitors don’t have complete remissions but these 2 drugs do have more targets right now. Keytruda did 21B in rev last year. Not a direct comparison bc Keytruda treats more cancers, but I think ctx110 will be *conservatively* a 1.5B/year drug. I can do more due diligence there as well.

These stocks have come down a lot since bubble times, crsp trades at a 3-4B market cap rn. Phase 2 trials of exacel ctx001 have shown 42/44 (last two patients became transfusion free after engraftment took.) Sickle cell disease is a challenging debilitating disease, with the rise of sickle cell centers in major cities people are able to live to their 55-65 now. Your red blood cells become misshapen with less oxygen. This misshapen aggregates of blood cells cause end organ damage, pain crisis (some docs think these patients are pain seeking), also another complications of an occlusive crisis is severe priapism 😊. These people are sick, sick, sick. Having a cure buys people a lot of life time**. ICER** a nonprofit that evaluates drug pricing and has been hard on drugs that don’t increase quality of life released a draft report on 4/13 (ICER says draft because they are awaiting more ctx001 data) say ctx001 can be priced around 1.9M for a one time cure, Martin in his YT videos put drug price around 1M, so someone in between I’m fine with for price.

Phase 3 results incoming and ctx001 approval in EU around 9/2023, being the first crisper drug approved for use in patients. NTLA has until 2027 for their approval of TTR amyloidosis. CRSP is also better because they understand the limitations of crisper 1.0, there might be higher fidelity gene editing crisper 2.0 coming don’t know much, but with ctx001 they are inserting and knocking out a functional gene (BCL11A) that regulates HbF. People who have this gene knocked out and also have sickle cell don’t have sickle crises, this was the idea behind this drug target. Knocking out a functional gene is easier right now with crisper tech than inserting a functional gene, this is part of my reasoning of why I think mgmt at crisper is better than most, they are also during multiplex crisper and trying to find ways to evade our immune system. Always smart to model nature to get shit done, imo.

**Draw backs of this company,** please watch Martin’s YT video on his thoughts on crisp, he mentions most. Vertex has a 60% share of the profit from ctx001. They bought the last 10% for 900mil going from 50 to 60%. This means Vertex values early stage development of ctx001 at 9B (900mil/10%). Mgmt for crisper is very subtle/smirking 😊 and underselling about demand in all earnings and gene therapy conferences , they also subtly mention being a 100B market cap.

I’m thinking 1K-3K patients in the first year (100K SCD population in the US). 1K\*1.5M \*.4 spilt = 600M for a company that trades at a 4B market cap. They already have former treated patients doing public relations/advertising for the drug, and family of treated patients buying all the stock they can.

Other drawbacks is doing the bone marrow transplant basically killing all bone marrow (marrow makes rbcs, white blood cells, and platelets), this is SERIOUS stuff where you are in the hospital for weeks to months until the autologous crisper edited marrow takes with the knocked out cells. Martin in his YT acknowledges this but he doesn’t realize how life threatening and shortening SCD is. The **ICER docs** do, and that’s why they price at 1.9M. Also another drawback is the CEO who I judge as running a tight ship sold 1M $ worth of shares and still has $17M worth.

I hope this helps give a better understanding of the stock CRSP, and why I think it’s severely undervalued in the market.