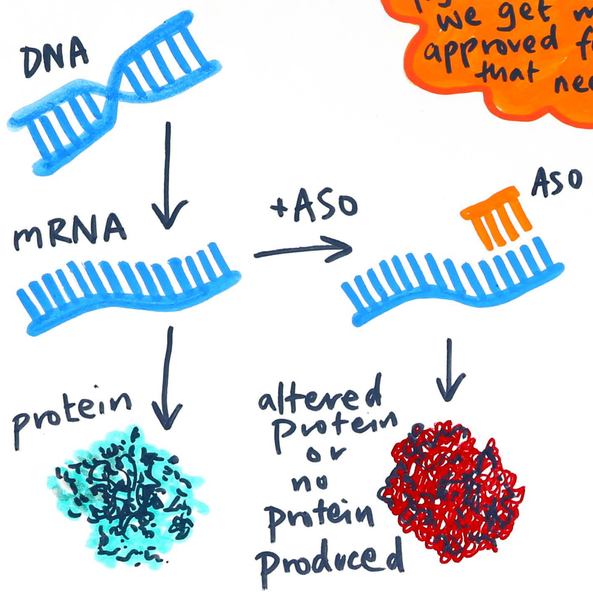
the RNA therapeutics revolution

So you have DNA being transcribed into RNA, in this case messenger RNA, & then translated into protein

ALEX FULTON



I'm trying to figure out why so we get more drugs approved for patients that need them

Some of the main drawbacks of RNA therapeutics is that they are not performing well in clinical trials ... causing liver toxicity

is liver toxicity a common thing to happen?

when you take a drug around 90% of this goes straight to liver, potentially overload — and not where you potentially want to target

are you testing on animals?

I don't work on animals. Animal testing is really not as useful as some people make out, especially with these kinds of therapies, because they are tailored to specific RNA in the human body

how expensive would these treatments be?

in short, very

it's the need that is the main drive with costs of these drugs

and really, it depends on the condition because some patients have really debilitating diseases that require constant drugs in order to treat, and if you're able to develop a RNA based treatment that can last 6 months or a year, with minimal dosing, although it's going to be expensive, it can end up being more cost effective compared to taking a pill everyday

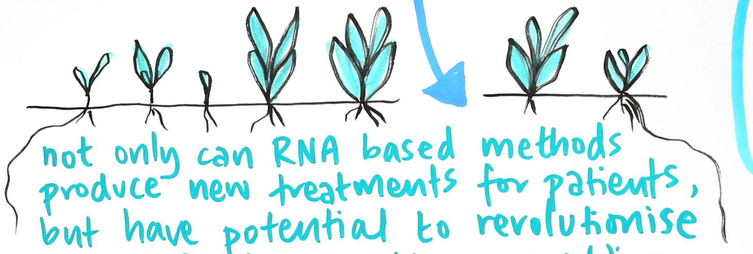
vs.

so you hit the source before it becomes a problem

the RNA therapeutics that I work on, such as **ASOs**, target this RNA & it leads to either degradation of the target RNA & no protein is produced, or it alters the protein that is produced to correct a misfolded protein which leads to a disease/condition, for example

we're only scratched the surface of developing drug treatments & 98% of all approved medicines, small molecule drugs, target at this protein level

the idea is we target the RNA level & the mRNA level instead of the protein...



not only can RNA based methods produce new treatments for patients, but have potential to revolutionise many fields e.g. with gene editing you're able to edit crops

only a matter of time before we develop much better therapies for more genetic conditions

RNA based drugs really are exciting because they alter this tiny change & have a BIG EFFECT

would it matter what time in life you started taking it

yes, for example, a disease where it gets worse over time, it's always best to treat early

many diseases are really life changing conditions that are due to issue with central nervous system. e.g. Duchenne muscular dystrophy & spinal muscular atrophy

what things are they being used for now?