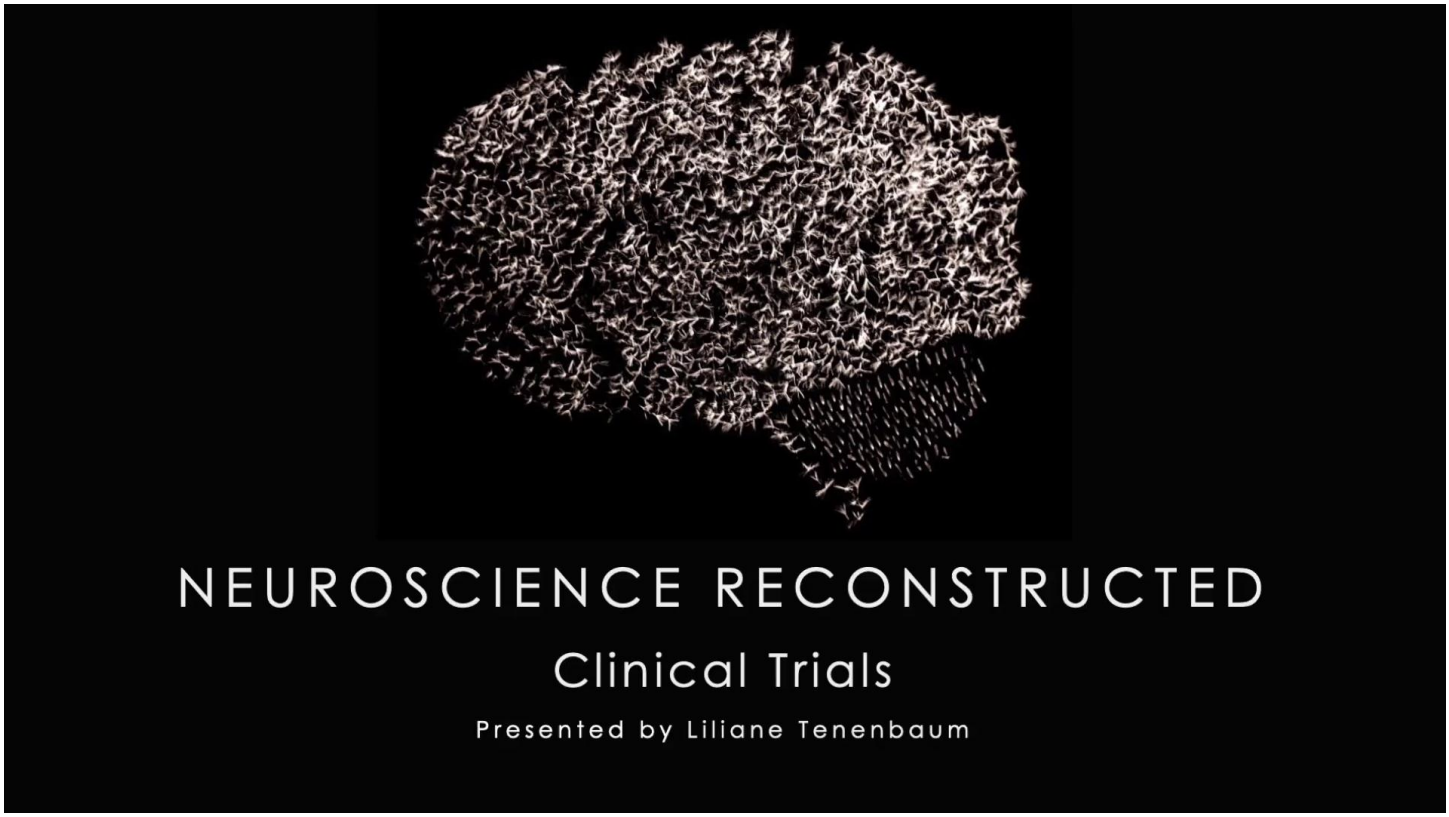


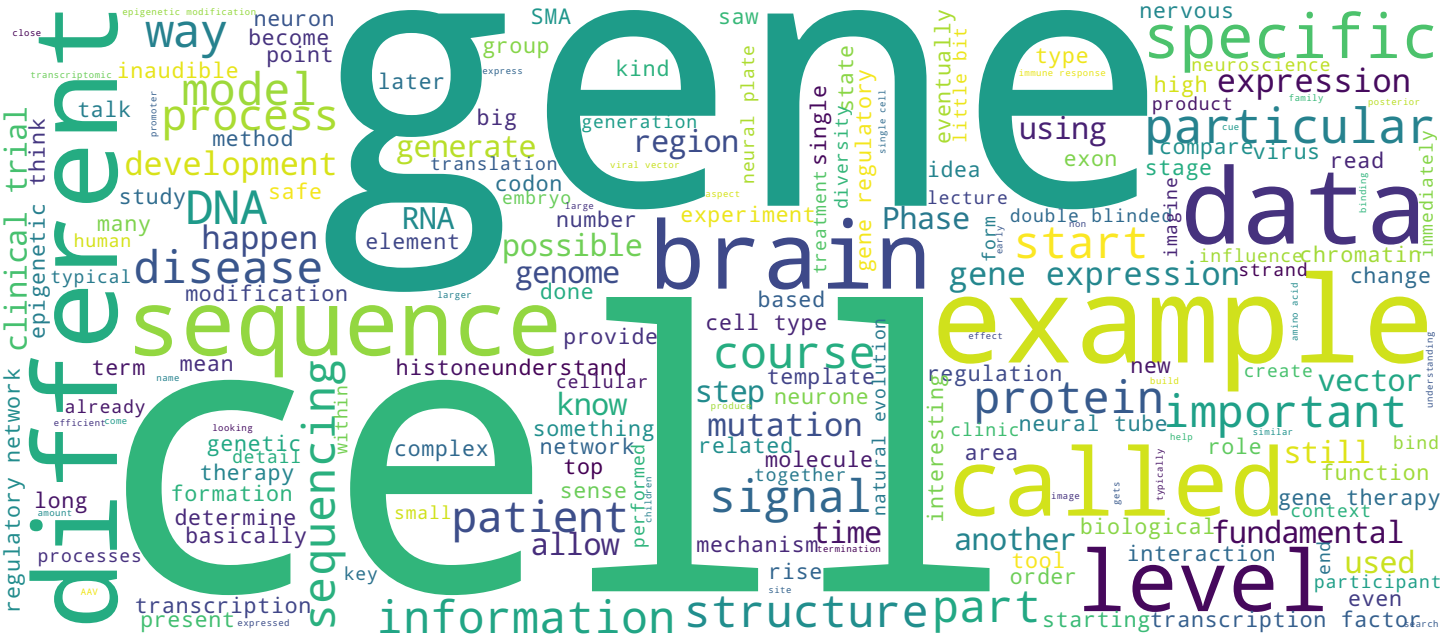


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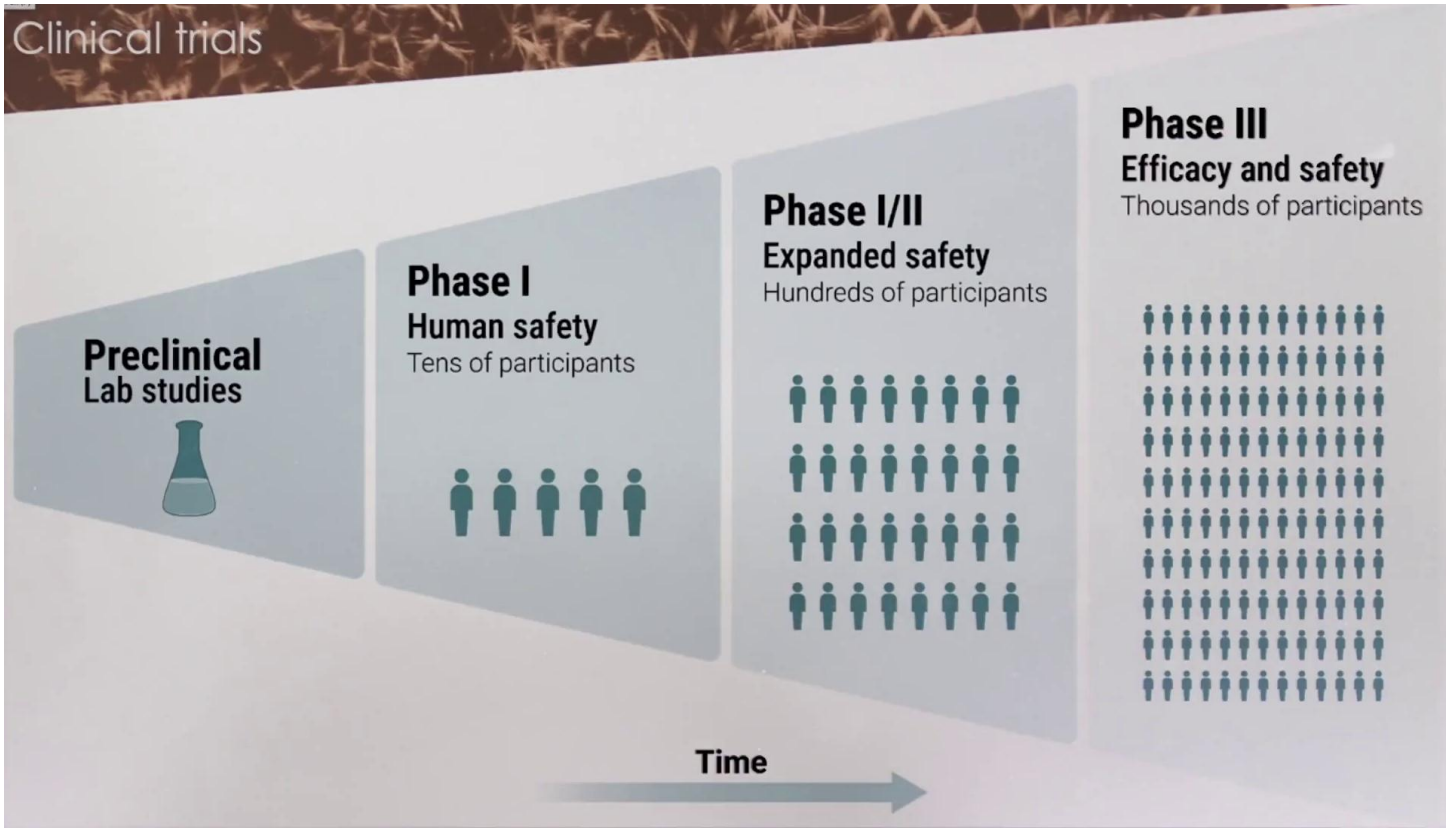
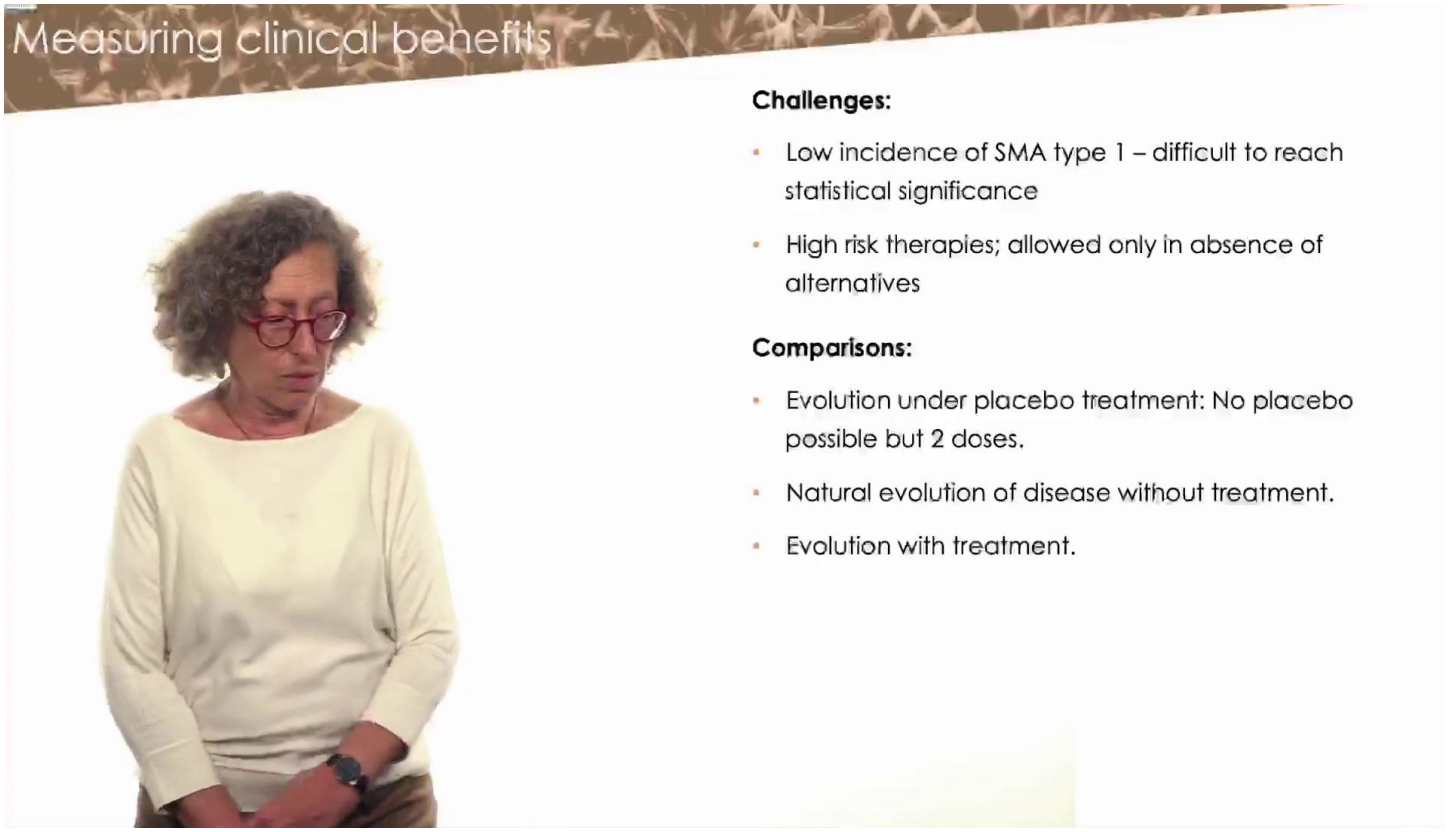



We can go to the clinics. This is also a very long process since there are several phases. First, you have to do a Phase 1, which is addressing only the safety and tolerability of the therapy. Then if it's okay, if it's safe, can make a larger one. Finally, when it has been demonstrated to be safe in a large amount of participants, you can finally go to Phase 3, and this theoretically, efficacy, clinical benefits are only evaluated in Phase 3. Now, you can immediately see that this is a problem for the rare diseases like SMA. Since we only have a few patients per million inhabitants, this making a Phase 3 trial with thousands of participants will not be possible. But since it is an incurable disease, some modification can be done to still be able to test this therapy for life-threatening diseases in smaller clinical trials.

Notes

Summary

0m 04s

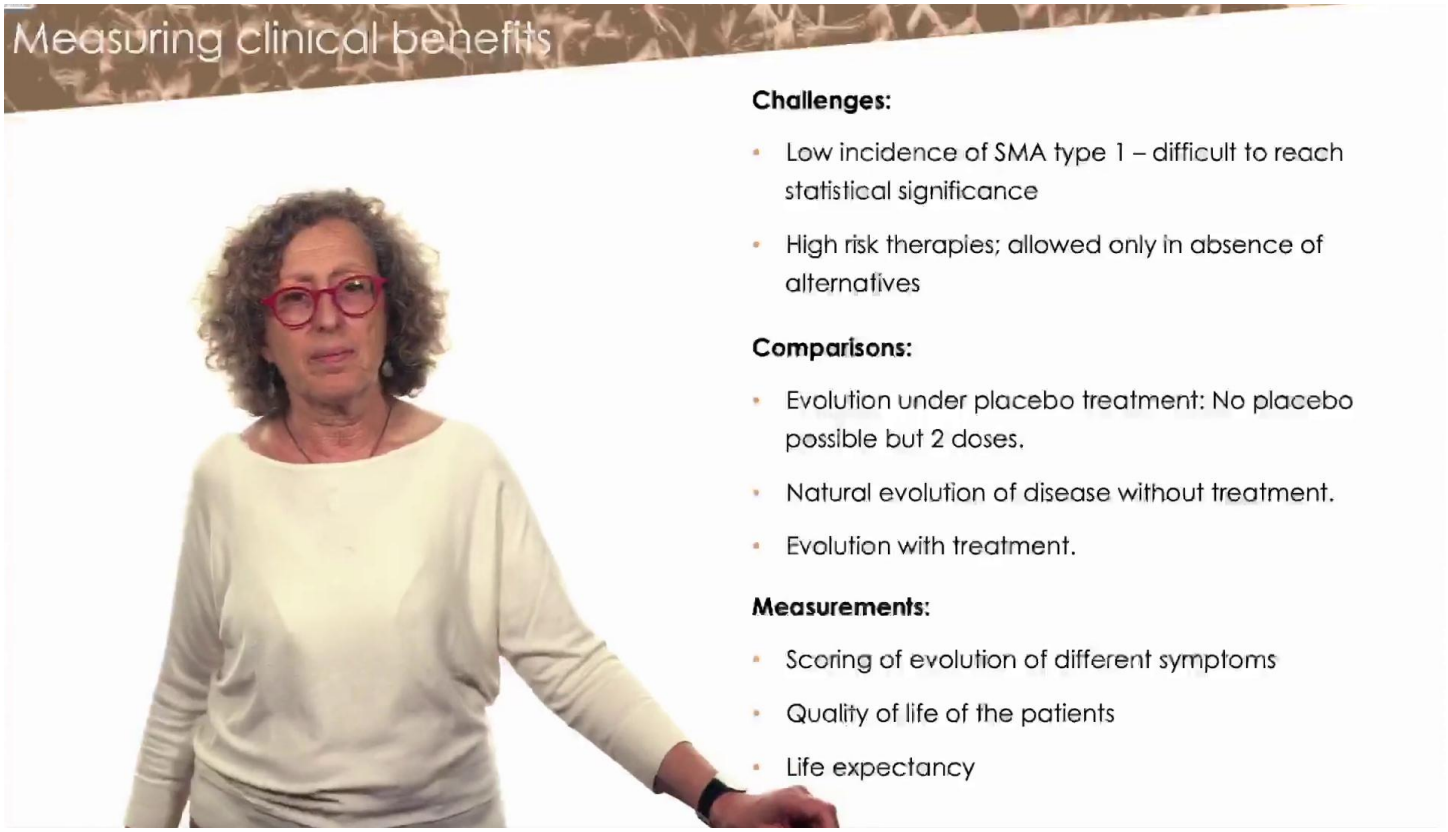



Since we don't have any alternative, there is no treatment. The clinical trial could still be performed, but with less patient. Another challenge is how to statistically evaluate the benefit. In Phase 3 clinical trials, you have to do what is called a double blinded placebo control protocol in which part of the patient are treated and others are not, and you compare the two group and neither the patient nor the clinician know that's why which one is in which group are particular participant. That's why it's called double blinded. In this case, it would be ethically not feasible to have a placebo group since the children will die soon if we don't do anything. To compensate for that, they did actually two different dose to evaluate whether the highest dose will be more beneficial than the low dose. Now, another way of proving that the therapy is efficient is to compare the evolution of the treated children with the natural evolution of the disease, which is very, very typical. It is known precisely when they start to need respiratory assistance and we also know that they are not able to sit and stand. Any improvement will be easily quantified compared to this natural evolution.

Notes

Summary

1m 36s




This before starting, you need to have a precise protocol for scoring the patient. This the different symptoms have been classified with different severity. Of course, the life expectancy is also something that can be quantified. These are the criteria that will allow to evaluate the success of the gene therapy.

Notes

Summary

3m 46s