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Development of N-of-1 RNA Therapies in the Netherlands and Europe

12PM, EST
April 29, 2022
Host: Dr. Gregory Costain

Register in advance for this webinar: https://us06web.zoom.us/webinar/register/WN_vXOheKQmTnOAG2Mt1BZbJw

Antisense oligonucleotides (AONs) offer the potential to treat patients with genetic diseases. Notably, for tissues allowing local injection, such as the brain and eye where high local exposure can be achieved with 3-4 infusions of low amounts of AONs annually. Proof-of-concept has been shown for example in spinal muscular atrophy and Leber congenital amaurosis. I will highlight the development of different AON treatment strategies with a focus on autosomal dominant ataxias. Then I will highlight how this approach can also benefit patients with private mutations, as was recently evidenced by the development of the custom-made AON milasen for a patient with Batten's disease. This underlines the potential of AONs as personalized medicines, specifically for patients with private mutations that are associated with brain or eye phenotypes. However, pharmaceutical companies are usually not interested in the development of such approaches, due to the extreme rarity of these variants.