



Gene Veritas

2 hrs ·

Roche's historic gene-silencing clinical trial for Huntington's disease is now ramping up, with the firm's scientists "actively thinking" about when and how to expand research to target groups beyond the current criterion of early- and mid-stage HD patients aged 25-65, said the program's scientific coordinator. "We're excited to be moving forward with the Phase 3 program," said Scott Schobel, M.D., M.S., Roche's associate group medical director and clinical science leader for the HD drug RG6042, in an interview with me. In the Phase 3 clinical trial, some groups are excluded, such as presymptomatic gene carriers like me (also known as prodromal or premanifest individuals) and juvenile Huntington's disease (JHD) sufferers, because of the need to first prove RG6042's efficacy in people where measurements can best be made and, in Dr. Schobel's words, "most likely to show an effect." "Though we do not have a planned prodromal trial, we are actively thinking about what that would look like, should the lead studies be supportive of pursuing that route," Dr. Schobel said. Similarly, for expanding to JHD and other age groups, "we're also having discussions." "That desire [to expand access] comes from a place of having seen and interacted a lot with the community and understanding the severe unmet need of [treating] juvenile HD, on the one hand, and also the highly compelling nature of preventing the decline from occurring in the first place, the ultimate goal of a Huntington's therapy," Dr. Schobel explained. However, at the moment, demonstrating RG6042's effectiveness in the Phase 3 trial is Roche's main goal. See this compelling, in-depth interview in my latest blog article.



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