

| Question Asked | Answer |
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| My kid is using Idebenone , alpha lipoic acid. Does patient need to stop using these medicines when patient starts using the medicine you are working on. Or now? Do they impact their progress? | Your child can take those medications during the clinical trial of AMX0035 in patients with Wolfram syndrome. it's important to note that we're still gathering safety data to include children in the trial. |
| What are results of Amylyx on wolfram animals? Also, how it compared to glp1? | Answered live |
| Is there an intention to transform Helios into a pill? Is there a marked difference compared to the results shown back then in phase 2 for ALS, that are indicating higher success for Wolfram? | Answered live |
| Is there a time limit for how long amx35 could be prescribed, or is it something that could be for life? | Answered live |
| When could the compound be available for patients overseas? | Answered live |
| Will AMX be available to UK as soon as it's available to the US? | That depends on the results of our ongoing trial and next step we take. |
| Have you started to look at safety of amx35 in younger than 17? | Answered live |
| Would the EU initiative exclude the UK? | The European Medicines Agency (EMA) based in the EU regulates the approval of medicines across the European Union, including the UK when it was a member. However, after Brexit, the UK established its own regulatory authority, the Medicines and Healthcare products Regulatory Agency (MHRA), which is responsible for approving drugs for the UK market .While the UK is no longer part of the EMA, it does have mechanisms for cooperation and alignment with the ENA on drug regulation. |
| Do you see a positive future for people with WS? | I do see a positive future for people with WS. We're actively developing safe and effective treatments for Wolfram syndrome, including oral medications, gene therapy, and regenerative therapy. Interim results of our ongoing clinical trial of AMX0035 in patients with Wolfram syndrome are encouraging, and other new treatments are on the horizon. I believe we are getting closer and closer to a cure. It will be a cure or a stabilization of the disease so patients can have a good quality of life. That's what I'm aiming at. |
| If there's any progress on gene editing, if it keeps being theory and animal studies or if it is pursued in some minor cases. | Our current plan involves initiating a safety and efficacy study in a small number of patients (n=10-12) with Wolfram syndrome. |
| Does that mean the expansion of the amx035 trials will be coming to the UK? If not, would we be able to get on the trials in Europe whilst living in the UK? | Dr Urano's answer - Our path forward hinges on the results we anticipate in the coming months and our ongoing interactions with the US FDA. Nothing is |

definitive at this stage. Your patience throughout this process is greatly valued.

Dr Timmons, Amylyx answer - The next trial of AMX0035 in Wolfram Syndrome has not been designed yet, so we don't have a final answer to your question just yet. Once Amylyx gets the results from the ongoing HELIOS trial, we will discuss the next trial design with Wolfram syndrome clinical experts, people with lived experience with Wolfram Syndrome, and also with regulatory authorities to determine the best study design. Your question touches on one key consideration for the next trial, which is where trial sites will be located. Amylyx considers a number of factors in deciding where trial sites are located, including the sites' expertise in running clinical trials in Wolfram Syndrome, convenience for participants to get to the sites, and how busy the site is with other trials and clinical care. There are potential sites in Europe, including the UK, that will be considered for the next Wolfram Syndrome trial. We will also take into account that some participants may not live close to a site, but will be willing to travel, when we design the trial as well.