Stanford Neuromuscular Program

SMA Program Announcements

August 16, 2016

We are happy to provide this update regarding SMA clinical trials for which Stanford is partnering with Biogen/Ionis, Cytokinetics, AveXis and Roche pharmaceutical companies.

Nusinersen developed by Ionis Pharmaceuticals & Biogen:
Because an interim analysis shows positive results, the pivotal trial of the drug **Nusinersen** in infants diagnosed with SMA1 was ended early so that all infants could receive treatment.

What Happens Next?

- All of the data regarding **Nusinersen** will be submitted to the FDA by the end of 2016 in attempt to get clinical approval, which we hope will be finalized within the next 12 months.
- Biogen is offering an Expanded Access Program (EAP) until the FDA finalizes its decision in which all individuals with SMA1 could receive Nusinersen. All sites considering participation in the EAP are working to answer outstanding questions about this EAP:

    - Even though the drug itself will be provided by Biogen without cost, insurance companies will have to pay the costs of treating patients with **Nusinersen** – which might cost $10,000 - $50,000 over the course of one year
    - 12 sites in the USA are eligible to participate in the EAP, and they are working to determine whether they have the capacity to participate, and how best to involve individuals equitably and ethically
    - The pivotal trial of **Nusinersen** for individuals with SMA2 will not be stopped early. Evaluation of participants is continuing, but we are not enrolling anyone new. We hope this trial will also show benefit, but we will not have those results for another year or so.

CK-107 developed by Cytokinetics
The orally administered drug **CK-107** is designed to increase muscle force while reducing fatigue – which will benefit almost everyone if it works the way we expect. The drug is designed to help everyone with SMA; to most quickly demonstrate the effect this trial is recruiting both ambulatory and non-ambulatory individuals with pre-specified functional abilities. We are happy to review criteria for participation with each interested family so we can involve those who are eligible. If you or your family member is living with SMA2 or SMA3, and you're interested in learning if you are eligible to participate in this exciting study, please let us know!

Future Clinical Trials:

AveXis

- Gene replacement therapy is moving forward, and we are hoping we can participate in a clinical trial in the near future. This has not yet been approved by the Review Board, which is our first step, but if we do participate it will be a very small study with, we imagine, very young individuals in the initial studies.

Roche

- Roche has a new orally administered compound that has been designed to significantly help all people with SMA. We are not sure yet which individuals we will study with this drug, or when the start date will be. As with all studies, it will first need to be reviewed by the Stanford Review Board, but we are hoping it might start in 2017.

It is certainly an exciting time in SMA research and clinical care, but we have a long way to go! If you are already in our Clinical Research Database we'll keep you posted as our research moves forward. For questions regarding any of the studies above, or to join our Research Database, please leave a message on our research line at (650) 725-4341 (option #2). Thanks for your interest, involvement and support for optimizing SMA clinical care and research – it is only by working together that we can effectively combat and overcome its effects.

Participant's rights questions, contact 1-866-680-2906.