

September 13, 2021

Dear XLMTM Patient Community,

On September 1, 2021, we announced a voluntary pause on all screening and dosing associated with the ASPIRO trial after a participant developed a serious adverse event. Despite aggressive medical interventions, the participant passed away on Thursday, September 9, 2021. The cause of death is still pending. We extend our deepest sympathies to the participant's family.

Astellas Gene Therapies is gathering the relevant clinical information and incorporating such information into the ongoing investigation regarding other serious adverse events observed to date in the ASPIRO trial.

Below please find additional background and our planned next steps:

- This was the first and only participant dosed following the December 23, 2020, release of the earlier FDA clinical hold.
- As communicated on September 1, Astellas Gene Therapies voluntarily paused screening and dosing.
- On September 3, 2021, the FDA informed Astellas Gene Therapies that the ASPIRO trial had been placed on clinical hold. Once we receive the official FDA clinical hold letter, we will review its contents and plan to have further dialogue with regulators on the path forward.
- We will investigate and review all findings with our independent data monitoring committee, our expert liver advisory panel and the ASPIRO site investigators.
- All currently enrolled participants will continue to be followed closely per the study protocol.

If your child is currently participating in the ASPIRO clinical trial, please speak with the physician and his or her staff at your clinical trial site if you have questions or would like to discuss the situation.

We are taking all necessary steps to understand these events and incorporate what we learn into the clinical development plan. Once again, we offer our deepest condolences to the participant's family and we remain committed to the development of AT132 and the XLMTM patient community.

Sincerely,

A handwritten signature in black ink that reads "Nathan Bachtell".

Nathan Bachtell, M.D., Senior Vice President and Head of Gene Therapy, Medical & Development