



November 16, 2018

USA Clinical Trials Update

We have a lot of new families in our community right now, so we'd like to take a little time to update you on the availability of the some of the clinical trials currently.

*There is an expansion of trials at **Abeona Therapeutics** for types A and B gene therapy that we have been continuous funders of and look forward to the results of the latest children receiving gene therapy in higher doses!*

The link to Abeona Type B > look for a few older patients in this trial

<https://clinicaltrials.gov/ct2/show/NCT03315182?cond=Sanfilippo+Syndrome&cntry=US&draw=2&rank=3>

Abeona Type A >

<https://clinicaltrials.gov/ct2/show/NCT02716246?cond=Sanfilippo+Syndrome&cntry=US&rank=7>

SOBI is currently running a trial for Type A out of Oakland, CA for 1- 6 years old that qualify. A few of our families are involved in this trial now.

<https://clinicaltrials.gov/ct2/show/NCT03423186?cond=Sanfilippo+Syndrome&rank=9>

Lysogene is also is gearing up in USA. Keep an eye on this trial for recruiting.

<https://clinicaltrials.gov/ct2/show/NCT03612869?term=Lysogene&cntry=US&rank=1>

Biomarin has a Treatment Study for Type B

<https://clinicaltrials.gov/ct2/show/NCT02754076?cond=Sanfilippo+Syndrome&rank=6>
(up to 10 years old)

These are trials that mostly younger children are eligible to apply for. We still don't have approved trials for a lot of older children yet! Hopefully we will see a few over 7-10 years old in some of these trials!

Please follow Team Sanfilippo on FB (TSF Inc.) for future announcements on other treatments, studies and trials coming soon!

<http://www.facebook.com/teamsanfilippo>

<http://www.teamsanfilippo.org>